income countries, factor replacement remains the only feasible option, in line with World Federation of Hemophilia (WFH) recommendations. **Conclusion:** Despite recent paradigm shifts in the treatment of pediatric hemophilia, factor replacement remains indispensable. Gene therapies hold promise for the future, but biological and ethical constraints currently prevent their application in children. Non–factor-based agents have facilitated prophylaxis but are insufficient in emergencies and lack long-term safety data, particularly in major surgical procedures and severe acute bleeding episodes. Factor replacement therapies, with their proven efficacy, predictable pharmacokinetics, established safety, and global accessibility, continue to stand as the gold standard treatment option for both today and the foreseeable future. *"A reference to a Turkish idiomatic saying, originally 'Introducing a new custom to an old village' (bringing new ways to an old place), which means introducing a revolutionary, unusual, or unexpected innovation or behavior into a traditional, clichéd order or way of doing things."

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Abstract 004

IRON CHELATİON İN MYELODYSPLASTİC SYNDROMES: WHO AND WHEN?

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Red blood cell (RBC) transfusions are the cornerstone of supportive care in patients with myelodysplastic syndromes (MDS). While transfusions alleviate symptomatic anemia, they inevitably lead to progressive iron accumulation in patients. This transfusional iron overload may exert toxic effects on the heart, liver, endocrine system, ultimately contributing to increased morbidity and mortality. Timely initiation of iron chelation therapy has become an important consideration in the comprehensive management of MDS. Chelation is primarily indicated for patients with lower-risk MDS (IPSS low or Int-1) who are expected to have longer survival, who remain transfusion-dependent. In such patients, iron overload not only threatens organ function also worsens prognosis. Multiple studies have shown that transfusion dependence is a negative prognostic factor, and retrospective analyses suggest that iron chelation may improve overall survival. Chelation is also particularly important in patients who are candidates for allogeneic stem cell transplantation, since excess iron has been associated with inferior transplant outcomes. By reducing systemic iron burden, chelation help optimize organ function and improve transplant eligibility. The decision is usually guided by transfusion history and serum ferritin levels. Most guidelines recommend considering chelation after approximately 20–30 units of RBC transfusions or when serum ferritin persistently exceeds 2500 ng/mL. The therapeutic goal is to maintain ferritin below 1000 ng/mL, minimizing iron-mediated oxidative stress and tissue damage. While serum ferritin is an imperfect surrogate, it remains a practical marker. More advanced techniques such as MRI T2* or SQUID can provide direct estimates of hepatic iron, but their availability is limited. Three chelators are currently in clinical use. Deferoxamine, administered subcutaneously or intramuscularly, is effective but limited by its parenteral route. Deferasirox, an oral once-daily agent, has become the preferred choice in many cases and is FDA-approved for transfusion-related iron overload. Randomized trials in lower-risk MDS demonstrated that deferasirox reduced ferritin, improved event-free survival, and even enhanced hematologic response in some patients. However, renal, hepatic toxicity require careful monitoring. Deferiprone, another oral agent, is mainly approved for thalassemia, can be considered when other chelators fail, though its use in MDS remains limited due to risk of agranulocytosis. Chelation has been associated with improved overall survival in observational studies, prospective trials provide encouraging evidence. Beyond survival, reversal of some iron-related cardiac, hepatic damage has been documented, underscoring its importance. Monitoring should include serial ferritin, renal, liver function, vigilance for adverse events. Individualization is critical: patients with advanced or high-risk MDS, limited life expectancy are less likely to benefit, and chelation is generally not recommended in such settings. Iron chelation therapy plays a vital role in selected MDS patients. It should be considered in lower-risk individuals with substantial transfusion requirements and elevated ferritin, especially in those with preserved organ function or who are candidates for transplantation. As evidence grows, iron chelation continues to evolve from a supportive measure into a prognostically meaningful intervention in the management of MDS.

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Abstract 005

THROMBOTIC THROMBOCYTOPENIC PURPURA (TTP)

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Thrombotic thrombocytopenic purpura (TTP) is a rare but life-threatening thrombotic microangiopathy caused by severe ADAMTS-13 deficiency due to either autoantibodies (immune TTP, iTTP) or biallelic mutations (congenital TTP, cTTP). The first International Society on Thrombosis and Haemostasis (ISTH) guidelines were issued in 2020. Since then, substantial advances in therapeutic strategies and real-world evidence have prompted an ISTH 2025 focused update. The most significant change relates to cTTP prophylaxis. A new strong recommendation was issued in favor of recombinant ADAMTS-13 (rADAMTS-13) over fresh frozen plasma (FFP) in patients in remission. This decision, supported by a phase 3 randomized crossover trial, demonstrated that rADAMTS-13 provides higher and sustained ADAMTS-13 activity and fewer TTP-related manifestations, with a favorable safety profile [1]. Where rADAMTS-13 is unavailable, the panel conditionally recommends FFP over a watch-and-wait strategy, shifting from the neutral stance in 2020 [1,2]. Pregnancy-related cTTP remains a high-risk setting, and prophylactic therapy—preferably rADAMTS-13, or intensified FFP when rADAMTS-13 is