Clotting factor concentrates for preventing bleeding and bleeding‐related complications in previously treated individuals with haemophilia A or B

The study of clotting factor concentrate prevention in people with hemophilia A or B found promising results in terms of its efficiency in controlling the illness. A systematic analysis of randomized controlled trials (RCTs) and quasi-RCTs found that preventive therapy significantly lowers bleeding frequency in previously treated persons with hemophilia compared to on-demand treatment. Furthermore, prevention has the potential to improve joint function, reduce discomfort, and improve overall quality of life in these individuals.

The comparison of several preventive regimens offered significant insights. While no significant variations in bleeding prevention were detected between dosing regimes, dose modification appeared to improve effectiveness. Notably, a twice-weekly regimen of clotting factor IX (FIX) showed a possible benefit in lowering total bleeds when compared to a once-weekly regimen of the same dose, highlighting the possibility of dose-dependent advantages.

Furthermore, adverse events linked to preventive were documented in certain trials, however they were few and usually unrelated to the study medicines, indicating a generally satisfactory safety profile. Importantly, no inhibitor development or blood-borne infections were discovered, strengthening the safety profile of this therapy strategy.

However, the evidence's confidence remains relatively restricted due to several biases that might have impacted the results. There is an urgent need for well-designed RCTs and prospective observational controlled trials with consistent definitions and measures to enhance these findings and create appropriate, cost-effective treatment regimens. Overall, the evidence highlights the significant potential of clotting factor concentrate preventive measures in reducing bleeding frequency and improving the well-being of people who have previously been treated for hemophilia A or B, while also emphasizing the need for more refined and conclusive studies to guide optimal treatment strategies.

REFERENCES

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