Clinical Evidence of Romiplostim in Aplastic Anemia: Korean Expert Recommendations

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Thrombopoietin receptor agonists (TPO-RAs) have emerged as an important therapeutic option in the management of aplastic anemia (AA), both as part of frontline treatment and in patients refractory to or relapsed after immunosuppressive therapy (IST). These agents stimulate hematopoietic stem and progenitor cells through activation of the thrombopoietin receptor, thereby promoting multilineage hematopoiesis. Clinical trials over the past decade have demonstrated that romiplostim can induce clinically meaningful hematologic responses in patients with AA previously treated with IST, with improvements observed across platelet, erythroid, and neutrophil lineages. In addition, recent studies incorporating romiplostim into combination strategies with standard IST have shown encouraging response rates and favorable safety profiles, suggesting a potential role in earlier lines of therapy.

Management of AA in Korea presents several unique clinical considerations that may influence therapeutic decision-making. Horse antithymocyte globulin is not available, and rabbit ATG is exclusively used in clinical practice, which is associated with deeper and more prolonged lymphocyte depletion. Furthermore, regulatory restrictions limit the maximum approved dose of eltrombopag in Korea compared with Western countries, potentially affecting treatment efficacy in some patients.

Importantly, romiplostim and eltrombopag activate the thrombopoietin receptor through distinct binding sites and signaling mechanisms. These mechanistic differences may explain why hematologic responses can still occur with romiplostim in patients who previously showed inadequate responses to eltrombopag. Accumulating clinical evidence suggests that sequential use or switching between TPO-RAs may provide meaningful therapeutic benefit in selected patients with AA.

This lecture will review the key clinical trials of TPO-RAs in AA, discuss mechanistic and clinical differences between available agents, and highlight practical treatment strategies with particular attention to the clinical and regulatory context in Korea.