Rigel Pharmaceuticals conducted a phase 2 open-label study to evaluate fostamatinib, an oral spleen tyrosine kinase (SYK) inhibitor, in warm antibody autoimmune hemolytic anemia (wAIHA). WAIHA is characterized by phagocytosis of antibody-bound red blood cells by macrophages in a SYK-dependent signaling pathway.

The phase 2 study enrolled 26 patients (25 evaluable) with wAIHA. Eligible adult patients who had primary or secondary wAIHA, hemoglobin (Hgb) <10g/dL, and a positive direct antiglobulin test, received fostamatinib at 150mg BID. The primary efficacy endpoint was attaining Hgb >10g/dL with an increase of ≥2g/dL from baseline by Week 24 in the absence of rescue therapy.

In the phase 2 study, fostamatinib markedly improved Hgb levels in 48% of 25 evaluable patients with wAIHA demonstrating the drug’s ability to mitigate red blood cell destruction in wAIHA through SYK inhibition. AEs were manageable and consistent with those previously reported with fostamatinib in other conditions.