REAL-WORLD OUTCOMES OF BELUMOSUDIL IN PATIENTS WITH CHRONIC GRAFT-VERSUS-HOST DISEASE: A SINGLE-CENTRE RETROSPECTIVE ANALYSIS

Easdale, Sandra[1]; Garnett, Catherine[2]; Nicholson, Emma[2]; Anthias, Chloe[2]; Ethell, Mark[2]; Spencer, Richard[2]; Dallas, Fiona[2]; James, Lisa[2]; Wilson, Beverley[2]; Wong, Jessica[2]

[1]The Royal Marsden NHS hospital, [2]The Royal Marsden NHS Hospital

Background: Chronic graft-versus-host disease (cGvHD) is a major cause of morbidity following allogeneic haematopoietic stem cell transplantation. Belumosudil, a ROCK2 inhibitor, has demonstrated efficacy in relapsed/refractory cGvHD, although real-world evidence remains limited. This is a single centre study evaluating the effectiveness and safety of belumosudil.

Methods: This retrospective observational study included adult patients with cGvHD treated with belumosudil between January 2024 and June 2025. The primary endpoint was overall response rate (ORR) according to NIH criteria. Secondary objectives included organ-specific responses and the impact of concurrent treatment with ruxolitinib and/or extracorporeal photopheresis (ECP).

Results: Forty-seven patients were included. Median age was 53 years (range 19–75), with 31 males and 16 females. Median time from transplant to belumosudil initiation was 20.5 months (range 3.0–93.6). At treatment initiation, cGvHD severity was mild in 1 patient, moderate in 16, and severe in 30. The overall response rate was 42.5%, increasing to 55.6% when patients who died within the first three months were excluded. Patients with one or two organs involved achieved a higher ORR than those with three or more organs affected (50.0% vs 33.3%). Complete responses were observed in oral (14.3%), liver (28.6%), gastrointestinal (20.0%), and skin (10.7%) cGvHD. Partial responses occurred across all organ systems, with the highest overall response rates in ocular (66.7%), skin (60.7%), and oral (57.2%) disease. ORRs were 64.3% for belumosudil plus ruxolitinib, 60.9% for belumosudil plus ECP, and 66.7% for triple therapy. Eleven deaths occurred during follow-up, seven (63.6%) within three months of treatment initiation; none were considered treatment-related.

Conclusions: Belumosudil demonstrated meaningful clinical activity across all major cGvHD organ systems in this real-world cohort. Responses were greatest among patients with less extensive disease, and combination therapy with ruxolitinib and/or ECP showed promising efficacy. Larger prospective studies are warranted to further evaluate these findings.