

(PS2244) SYSTEMIC TREATMENT WITH THE ROCK2 INHIBITOR BELUMOSUDIL IN OCULAR CHRONIC GRAFT VERSUS HOST DISEASE

Topic: 23. Stem cell transplantation - Clinical

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Background

Chronic graft-versus-host disease (cGVHD) is the most common complication following allogeneic hematopoietic stem cell transplantation (allo-HSCT). Ocular involvement (oGVHD) occurs in 30%–85% of cGVHD patients and significantly diminishes vision and quality of life, and is associated with increased non-relapse mortality (NRM). Despite advances in understanding its pathogenesis, therapeutic progress for oGVHD has remained limited in recent years, underscoring a significant unmet clinical need.

Aims

To evaluate the efficacy of oral belumosudil on ocular surface disease in patients with steroid-refractory chronic graft-versus-host disease (SR-cGVHD).

Methods

In this retrospective study, we analyzed patients with SR-cGVHD and ocular involvement (oGVHD) treated with belumosudil (200 mg daily). Ocular assessments at baseline and 6 months included the International Chronic Ocular GVHD Consensus Group severity score, NIH eye score (0-3), corneal fluorescein staining (CFS, 0-4), tear break-up time (TBUT), Schirmer's I test, and the Ocular Surface Disease Index (OSDI). Treatment response rates were assessed per NIH consensus criteria.

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Results

Among 78 patients with SR-cGVHD treated with belumosudil, 40 had oGVHD, with a baseline NIH eye score of 2 (1–3). Of these, 28 patients completed both baseline and 6-month assessments. The cohort had high-risk features: 50.0% (14/28) had severe (NIH grade) systemic disease and 82.1% (23/28) had conjunctival fibrosis.

At 6 months, the oGVHD severity score improved in 53.6% (15/28), was stable in 35.7% (10/28), and progressed in 10.7% (3/28). The OSDI improved in 64.3% (18/28), was stable in 25.0% (7/28), and worsened in 10.7% (3/28).

Objective signs also improved from baseline: median CFS decreased from 4 (1–4) to 3 (1–4); median TBUT increased from 1.00 s (0.8–4.8) to 2.00 s (0.0–4.0); and median ST improved from 1.0 mm (0.0–5.0) to 2.0 mm (0.0–22.0).

A greater ocular benefit was observed in the 53.6% (15/28) of patients who achieved systemic overall response, with 66.7% (10/15), showing improved oGVHD severity scores and 80.0% (12/15) reporting better OSDI. Objective signs also improved in this subgroup: median CFS decreased from 4 (1–4) to 3 (1–4); median TBUT increased from 1.00 s (0.0–4.0) to 2.00 s (0.0–4.0); and median ST improved from 1.0 mm (0.0–2.0) to 2.0 mm (0.0–6.0).

Visual acuity remained stable, and 42.9% (12/28) of patients achieved corticosteroid dose reduction. Treatment was well-tolerated.

Variables	N= 28
Age, median(range),y	39 (19-64)
male	11 (39.3%)
Disease, n(%)	
AML	15 (53.6%)
ALL	6 (21.4%)
MDS	5 (17.9%)
Others	2 (7.1%)
Donor type, n(%)	
MSD	7 (25.0%)
HRD	16 (57.1%)
URD	5 (17.9%)
Conditioning intensity, n(%)	
MAC	24 (85.7%)
RIC	4 (14.3%)
Median time of cGVHD diagnosis from transplantation	251 (93-898) days
NIH cGVHD severity at the strat of belumosudil, n(%)	
Severe	n=14 (50.0%)
Moderate	n=14 (50.0%)
Median NIH oGVHD eye score before belumosudil treatment	2 (1-3)
Median oGVHD severity score before belumosudil treatment	9.5 (6-10)
Median CFS grade score before belumosudil treatment	4 (1-4)
Median modified eye Lee symptom scale before belumosudil treatment	6.5 (2-12)
Median corticosteroid dose (mg) before belumosudil treatment	0 (0-40)

Table1 Baseline patient demographics and clinical characteristics

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Summary/Conclusion

Systemic belumosudil was associated with improvements in ocular surface parameters and symptoms in steroid-refractory oGVHD, with favorable tolerability. Ocular improvement was closely linked to systemic response, warranting prospective validation.

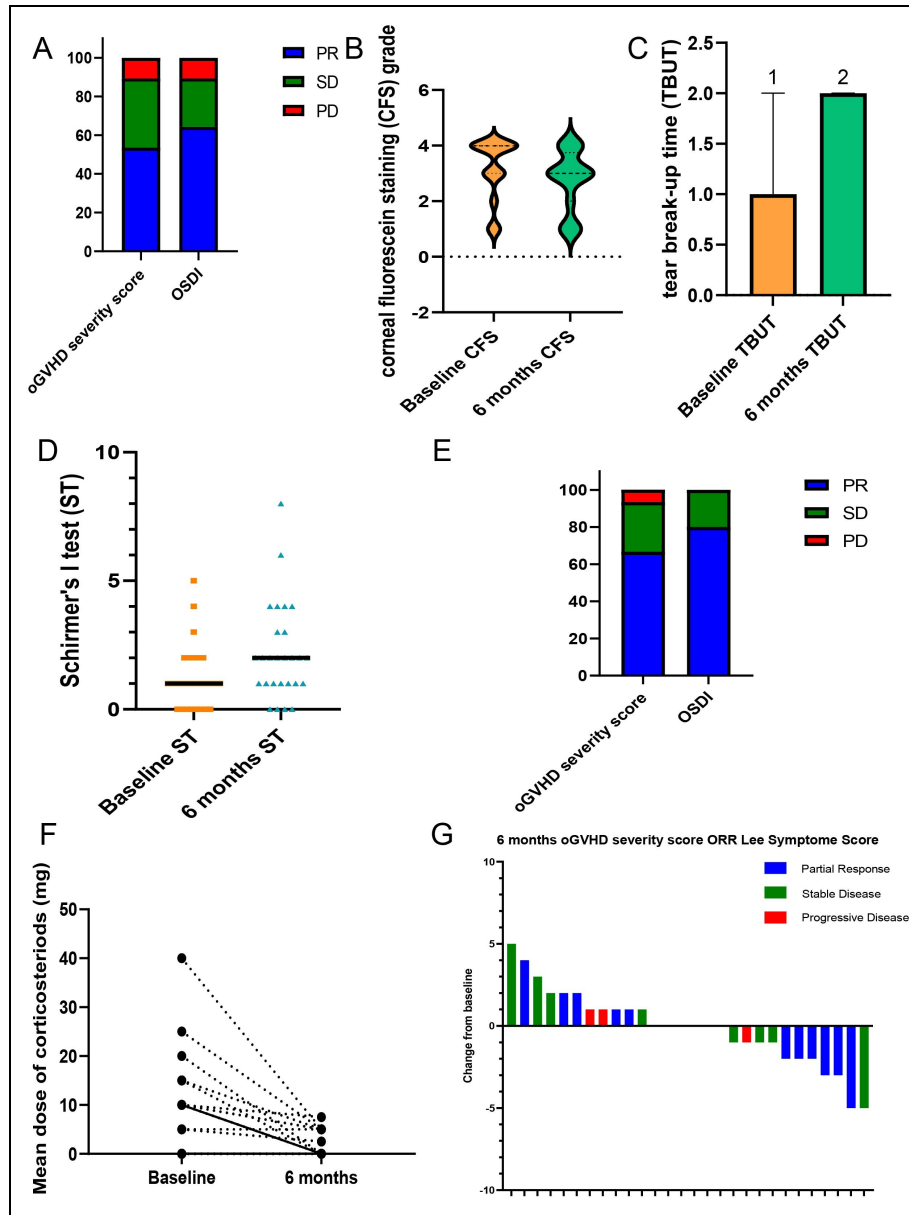


Figure 1. The efficacy of Belumosudil for oGVHD (A) The treatment response based on oGVHD severity score and OSDI. (B) The baseline and 6-months after belumosudil therapy of CFS grade. (C) The baseline and 6-months after belumosudil therapy of TBUT. (D) The baseline and 6-months after belumosudil therapy of Schirmer's Test. (E) The treatment response based on oGVHD severity score and OSDI in systemic ORR patients. (F) The baseline and 6-months after belumosudil therapy of Corticosteroid reduction. (G) The change of Lee Symptoms Score change at 6-months belumosudil therapy from baseline.

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