(PS2231) BELUMOSUDIL IN PEDIATRIC PATIENTS WITH STEROID-REFRACTORY CHRONIC GRAFT-VERSUS-HOST DISEASE

Topic: 23. Stem cell transplantation - Clinical

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Background

Belumosudil has been introduced to treat steroid-refractory chronic graft-versus-host disease (SR-cGVHD) in adult patients. Similarly, cGVHD occurred in nearly 25% of pediatric allogeneic hematopoietic cell transplant (HCT) recipients. But the lack of data in pediatric SR-cGVHD patients contributes to poor prognosis, especially in young children aged<12.

Aims

We aim to investigate the efficacy and safety of belumosudil in pediatric patients.

Methods

This study included 49 HCT pediatric patients with SR-cGVHD failing multiple lines of treatment (LOTs), and all patients received belumosudil. The primary endpoint was the overall response rate (ORR), failure-free survival (FFS), and overall survival (OS), and adverse effects (AEs) during belumosudil administration were documented.

Results

The median time to response was 6 weeks (range, 3-18), and the ORR was 81.6%, and 38.7% of patients achieved CR. The best ORR was also assessed in other affected organs, and organ-specific analyses demonstrated an ORR of 93.1% in the skin, 90.0% in the mouth, 90.9% in the liver, 87.5% in the eyes, and 94.9% in the gastrointestinal (GI) tract. The overall FFS and OS were 77.0% (95% CI, 70.1-83.9) and 100% in patients who received belumosudil. Belumosudil was well tolerated, and the AE rates were low in patients with belumosudil.

Summary/Conclusion

Belumosudil was effective and well-tolerated in pediatric patients with SR-cGVHD after failing multiple LOTs as salvage therapy, and most included patients reduced the dose or discontinued using corticosteroids and other immunosuppressive regimens to minimize their side effects and cost burden.

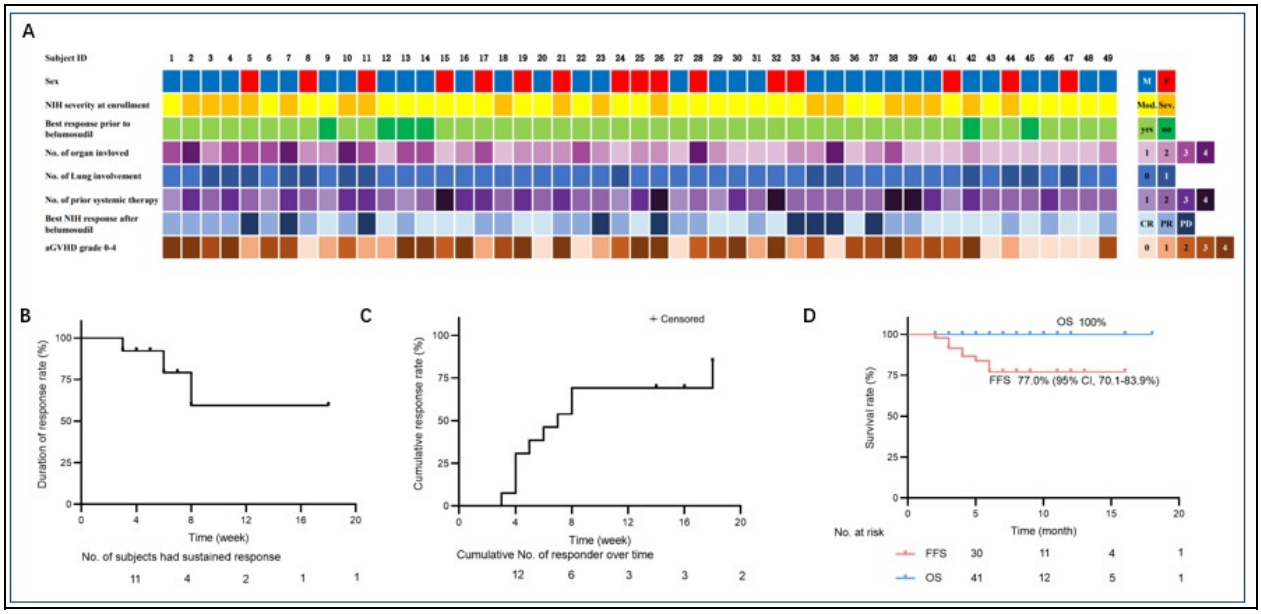

Figure A. Heatmap of disease status and best changes in response metrics in all patients included in the belumosudil group. Baseline characteristics for all 13 subjects and the best improvement of overall response were illustrated. aGVHD, acute graft-versus-host disease; M, male; F, female; Mod, moderate; Sev, severe; PR, partial response; SD, stable disease; PD, progression disease. B. Kaplan-Meier plot of duration of response rate of the belumosudil responder population; C. The time to respond to the belumosudil among responders. DOR, duration of response. D. Kaplan-Meier curves of estimated FFS and OS in patients received belumosudil.