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abstract

Belumosudil is effective in Pediatric Patients with chronic Graft-versus-Host disease after failed Ruxolitinib treatment

Yuanyuan Chen, Xiaojun Xu, Fenfen Jin

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Belumosudil is effective in Pediatric Patients with chronic Graft-versus-Host disease after failed Ruxolitinib treatment

Author: Yuanyuan Chen ¹, Xiaojun Xu, Fenfen Jin

Affiliation: ¹ Department of Pediatric Hematology-Oncology, Children's Hospital of Zhejiang University School of Medicine

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Introduction: Chronic graft-versus-host disease (cGVHD), including bronchiolitis obliterans syndrome (BOS), are some devastating outcomes in pediatric patients who received allogeneic hematopoietic cell transplant (HSCT). Belumosudil, an oral selective inhibitor of Rho-associated coiled-coil-containing protein kinase 2 (ROCK2), shows excellent therapeutic effect of BOS and can reverse pulmonary fibrosis.

Methodology: Seven patients who had been treated with at least one line of systemic therapy and ruxolitinib in China were enrolled in this study from April, 2024 to November, 2024. Organs affected by cGVHD at the time of initiating Belumosudil, including lung, skin, liver, eyes, mouth, esophagus, gastrointestinal tract, and joint/fascia, were evaluated by Lee Symptom Scale (LSS) score.

Results: All of the 7 patients were male. Their age and weight were shown as following: 12-year old (28kg), 12-year old (21.5kg), 8-year old (19kg), 6-year old (16.8kg), 5-year old (14kg), 2-year old (8.9kg), and 1-year old (9.8kg). Belumosudil 200 mg once daily was taken in cGVHD patients older than 6-year old. For patients who were younger than 6-year old, the dosage of Belumosudil was adjusted as follows: 66 mg once daily with weight 10-15kg, and 50mg once daily with weight less than 10kg. The median number of organs affected by cGVHD at the time of initiating Belumosudil was 3 (range, 1-5), and the median number of LSS scores was 6 (range, 1-15). The most commonly affected organs were lungs (n=7). After 8 weeks of Belumosudil treatment, all patients showed remarkable improvement in their Chest CT Scans and pulmonary function.

Conclusion: Our study suggests that Belumosudil can be well-tolerated in pediatric cGVHD patients who failed ruxolitinib treatment, providing a remarkable improvement of pulmonary function and encouraging quality of life.