Bone Marrow Transplantation

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Modified umbilical cord-blood transplantation for pediatric patients with mucopolysaccharidosis

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Mucopolysaccharidoses (MPSs) are a group of inherited errors of metabolism caused by gene encoding mutation that affects the degradation of mucopolysaccharides or glycosaminoglycans (GAGs), resulting in the accumulation of glycosaminoglycans in various organs and tissues of patients with MPS leads to multi-systemic clinical picture with a broad range of clinical signs and symptoms [1]. Although the advent of enzyme replacement therapy (ERT) has paved the way for MPS treatment, the blood-brain barrier (BBB) has prevented patients with central nervous system manifestations from benefiting from ERT [2]. Therefore, allogenic bone marrow transplantation (HSCT) is still the only effective treatment option for patients with MPS [3, 4].

This retrospective study included 60 patients with MPS who underwent a new conditioning regimen based on a slightly modified position of ATG or the first use of

Access options

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Contributions

YY, GQ, YS, and XS designed, wrote, and revised the manuscript. YY, GQ, ZL, YZ, YS, XZ, XQ, FJ, SF, JC collected data and provided clinical care. YY, GQ, and JQ analyzed the clinical data. All authors approved the final manuscript for publication.

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Ethics declarations

Competing interests

The authors declare no competing interests.

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