



[nature](#) > [bone marrow transplantation](#) > [correspondence](#) > article

Correspondence | Published: 26 October 2022

## Modified umbilical cord-blood transplantation for pediatric patients with mucopolysaccharidosis

[Yan Yue](#), [Guangming Qiao](#), [Zhouyang Liu](#), [Yaochen Zhang](#), [Yajuan Shi](#), [Xuexin Zhang](#), [Xiaoyu Qi](#), [Fan Jiang](#), [Shifen Fan](#), [Jiao Chen](#), [Jiayue Qin](#), [Yuan Sun](#)  & [Xiaodong Shi](#) 

[Bone Marrow Transplantation](#) **58**, 112–114 (2023)

**196** Accesses | [Metrics](#)

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 on the first day of

This website sets only cookies which are necessary for it to function. They are used to enable core functionality such as security, network management and accessibility. These cookies cannot be switched off in our systems. You may disable these by changing your browser settings, but this may affect how the website functions. Please view our privacy policy for further details on how we process your information. Dismiss

---

This is a preview of subscription content, [access via your institution](#)

---

## Access options

---

Access to this article via **China Institute of Science & Technology acting through National Science and** is not available.

[Change institution](#)

### Buy this article

- Purchase on SpringerLink
- Instant access to full article PDF

[Buy now](#)

### Subscribe to this journal

Receive 12 print issues and online access

**251,40 € per year**

only 20,95 € per issue

[Learn more](#)

Prices may be subject to local taxes which are calculated during checkout

### Additional access options:

- [Log in](#)
- [Learn about institutional subscriptions](#)
- [Read our FAQs](#)
- [Contact customer support](#)

### Data availability

This website sets only cookies which are necessary for it to function. They are used to enable core functionality such as security, network management and accessibility. These cookies cannot be switched off in our systems. You may disable these by changing your browser settings, but this may affect how the website functions. Please view our privacy policy for further details on how we process your information.

## References

---

1. Priller J, Flugel A, Wehner T, Boentert M, Haas CA, Prinz M, et al. Targeting gene-modified hematopoietic cells to the central nervous system: use of green fluorescent protein uncovers microglial engraftment. *Nat Med*. 2001;7:1356–61.

---

2. Sato Y, Okuyama T Novel enzyme replacement therapies for neuropathic mucopolysaccharidoses. *Int J Mol Sci* 2020; 21. e-pub ahead of print 2020/01/16; <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.3390/ijms21020400>.

---

3. Krivit W. Allogeneic stem cell transplantation for the treatment of lysosomal and peroxisomal metabolic diseases. *Springer Semin Immunopathol*. 2004;26:119–32. <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.1007/s00281-004-0166-2>.

---

4. Malatack JJ, Consolini DM, Bayever E. The status of hematopoietic stem cell transplantation in lysosomal storage disease. *Pediatr Neurol*. 2003;29:391–403. <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.1016/j.pediatrneurol.2003.09.003>.

---

5. Noh H, Lee JI. Current and potential therapeutic strategies for mucopolysaccharidoses. *J Clin Pharm Ther*. 2014;39:215–24. <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.1111/jcpt.12136>.

---

6. Politikos I, Davis E, Nhaissi M, Wagner JE, Brunstein CG, Cohen S, et al. Guidelines for cord blood unit selection. *Biol Blood Marrow Transpl*. 2020;26:2190–6. <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.1016/j.bbmt.2020.07.030>. e-pub ahead

This website sets only cookies which are necessary for it to function. They are used to enable core functionality such as security, network management and accessibility. These cookies cannot be switched off in our systems. You may disable these by changing your browser settings, but this may affect how the website functions. Please view our privacy policy for further details on how we process your information.

hurler syndrome. Biol Blood Marrow Transpl. 2009;15:618–25. <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.1016/j.bbmt.2009.01.020>.

---

8. Boelens JJ, Aldenhoven M, Purtill D, Ruggeri A, Defor T, Wynn R, et al. Outcomes of transplantation using various hematopoietic cell sources in children with Hurler syndrome after myeloablative conditioning. Blood. 2013;121:3981–7. <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.1182/blood-2012-09-455238>.
- 

9. Aldenhoven M, Wynn RF, Orchard PJ, O'Meara A, Veys P, Fischer A, et al. Long-term outcome of Hurler syndrome patients after hematopoietic cell transplantation: an international multicenter study. Blood. 2015;125:2164–72. <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.1182/blood-2014-11-608075>.
- 

10. Wang J, Luan Z, Jiang H, Fang J, Qin M, Lee V, et al. Allogeneic hematopoietic stem cell transplantation in thirty-four pediatric cases of Mucopolysaccharidosis-A ten-year report from the China children transplant group. Biol Blood Marrow Transpl. 2016;22:2104–8. <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.1016/j.bbmt.2016.08.015>.
- 

11. Parini R, Deodato F Intravenous enzyme replacement therapy in mucopolysaccharidoses: clinical effectiveness and limitations. *Int J Mol Sci* 2020; 21. e-pub ahead of print 2020/04/29; <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.3390/ijms21082975>.
- 

## Acknowledgements

This website sets only cookies which are necessary for it to function. They are used to enable core functionality such as security, network management and accessibility. These cookies cannot be switched off in our systems. You may disable these by changing your browser settings, but this may affect how the website functions. Please view our privacy policy for further details on how we process your information.

## Authors and Affiliations

### **Department of Hematology and Oncology, Hospital of Beijing Jingdu Pediatrics, Beijing, China**

Yan Yue, Zhouyang Liu, Fan Jiang, Shifen Fan, Jiao Chen & Yuan Sun

### **Department of Hematology and Oncology, Hospital of Hebei Pediatrics of Integrated Traditional Chinese and Western Medicine, Hebei, China**

Guangming Qiao, Yaochen Zhang, Yajuan Shi, Xuexin Zhang & Xiaoyu Qi

### **Department of Medical Affairs, Acornmed Biotechnology Co., Ltd., Tianjin, China**

Jiayue Qin

### **Department of Hematology and Oncology, Hospital of Capital Institute of Pediatrics, Beijing, China**

Xiaodong Shi

## 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.

## Corresponding authors

Correspondence to [Yuan Sun](#) or [Xiaodong Shi](#).

## Ethics declarations

---

## Competing interests

The authors declare no competing interests.

## Additional information

---

**Publisher's note** Springer Nature remains neutral with regard to jurisdictional claims in published maps and institutional affiliations.

This website sets only cookies which are necessary for it to function. They are used to enable core functionality such as security, network management and accessibility. These cookies cannot be switched off in our systems. You may disable these by changing your browser settings, but this may affect how the website functions. Please view our privacy policy for further details on how we process your information.

## Rights and permissions

---

[Reprints and permissions](#)

## About this article

---

Cite this article

Yue, Y., Qiao, G., Liu, Z. *et al.* Modified umbilical cord-blood transplantation for pediatric patients with mucopolysaccharidosis. *Bone Marrow Transplant* **58**, 112–114 (2023). <https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.1038/s41409-022-01858-5>

### Received

18 April 2022

### Revised

11 October 2022

### Accepted

14 October 2022

### Published

26 October 2022

### Issue Date

January 2023

### DOI

<https://doi-org-s-65.lib1-fx.fjmu.edu.cn/10.1038/s41409-022-01858-5>

Subjects

[Haematological cancer](#)

• [Translational research](#)

**Bone Marrow Transplantation (*Bone Marrow Transplant*)** | ISSN 1476-5365 (online) | ISSN 0268-3369 (print)

This website sets only cookies which are necessary for it to function. They are used to enable core functionality such as security, network management and accessibility. These cookies cannot be switched off in our systems. You may disable these by changing your browser settings, but this may affect how the website functions. Please view our privacy policy for further details on how we process your information.