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Title: Allogeneic stem cell transplantation: a curative treatment for paroxysmal nocturnal hemoglobinuria with PIGT mutation

Provenance and peer review: Unsolicited Manuscript; Externally peer reviewed

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Reviewer’s code: 02536364

Position: Peer Reviewer

Academic degree: MD, PhD

Professional title: Doctor

Reviewer’s Country/Territory: Japan

Author’s Country/Territory: France

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Reviewer chosen by: AI Technique

Reviewer accepted review: 2021-11-30 10:16

Reviewer performed review: 2021-12-02 14:03

Review time: 2 Days and 3 Hours

Scientific quality

[ Y] Grade A: Excellent  [ ] Grade B: Very good  [ ] Grade C: Good
[ ] Grade D: Fair  [ ] Grade E: Do not publish

Language quality

[ ] Grade A: Priority publishing  [ Y] Grade B: Minor language polishing
[ ] Grade C: A great deal of language polishing  [ ] Grade D: Rejection

Conclusion

[ Y] Accept (High priority)  [ ] Accept (General priority)
[ ] Minor revision  [ ] Major revision  [ ] Rejection

Re-review

[ Y] Yes  [ ] No
SPECIFIC COMMENTS TO AUTHORS
This case is probably the first case in the world where allo-SCT was performed to treat an extremely rare PNH case caused by a mutation in PIGT, not PIGA. I am very grateful and excited that I was able to read such a valuable case report before it was released to the world. I think this paper will be cited many times in the future when similar cases are reported. Therefore, in order to further increase the educational value, please add the following two points to the discussion. ① In common PNH caused by mutation of PIGA, there is a risk of developing GVHD, so it is difficult for even young people to perform allo-SCT. (At least I think so, and I've been taking eculizumab, now ravulizumab, on a regular basis for years without transplanting to my patient.) Did you decide to transplant PIGT-PNH this time because it was thought that the biological response after transplantation could be fundamentally different from that of PNH with PIGA mutations? ② I would like to ask why you switched to transplantation, even though eculizumab was effective. It is also related to ①, please tell us the points that will not cause GVHD. I hope that you will explain the above two points and that this paper will be published to the world as soon as possible.