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Time needed to complete: 39m

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Let's Discuss Transplantation

Announcer:

Welcome to CME on ReachMD. This episode is part of our MinuteCME curriculum.

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Dr. Andemariam:

This is CME on ReachMD, and I'm Dr. Biree Andemariam. Allogeneic hematopoietic stem cell transplantation, or HSCT, can cure sickle cell disease. Let's learn more about how and when hematopoietic stem cell transplantation is used in the management of sickle cell disease.

So what is a hematopoietic stem cell transplantation? Well, it's transplanting blood stem cells from a donor without sickle cell disease into a recipient who does have sickle cell disease. The first thing you need to do is to identify a suitable donor, and that's most commonly an HLA identical sibling. Other donor cells can come from unrelated match donors, partially matched or half-matched family members, or in some circumstances, from an umbilical cord blood donor. The blood cells are harvested from the donor and stored, and then the sickle cell disease patient is given high doses of chemotherapy to get rid of all the remaining blood cells in the bone marrow in order for them to be able to receive the healthy donated cells. The cells from the donor which are healthy are then infused into the sickle cell patient through a simple IV. The cells are remarkable in that they can find their way into the recipient's bone marrow, where they begin to divide and repopulate the patient's bone marrow as his or her own. This essentially cures the patient of sickle cell disease. But unfortunately, only about 20% of patients with sickle cell disease in the United States have a matched donor.

It's also important to think about what patients are appropriate for hematopoietic stem cell transplantation and to understand what some of the donor requirements are. And there's a bit of debate here. So the goal is to transplant someone with sickle cell disease before they become too sick, particularly before they develop significant organ damage, such as kidney disease, liver disease, or heart disease, that might even preclude their ability to safely undergo the rigors of a transplant. However, since there are transplant-related risks such as graft versus host disease and life-threatening infection, as well as organ toxicity, one could likely not be transplanted if their sickle cell disease was not under really good control or if they had minimal disease manifestations.

Now what about the donor? So we know that the most ideal match is a full sibling (same mom, same dad) who is also a full bone marrow match. Ideally, the sibling is a perfect 10 out of 10 match. And if a full sibling match can't be found, it's also possible to transplant hematopoietic stem cells from a donor who's only a half match. That's something that we call a haploidentical transplant.

There are different risks associated with this. And it's really best to consult a transplant expert to evaluate which is best for a particular patient. The main requirement for a donor is to not have sickle cell disease or infection, such as HIV or hepatitis C. But what's important to know is that it's perfectly fine for the donor to have sickle cell trait. And this is commonly the case in siblings.

So what are the realities and clinical outcomes associated with hematopoietic stem cell transplantation in sickle cell disease? Well, the reality is that most patients who are transplanted are younger, typically under age 16 years. And the median age for transplantation is around 9 years of age. And that's because we need generally healthier patients in order for them to meet the requirements to undergo transplantation.

What about the outcomes? Well, 5-year survival is really good. It's 95% for patients under 16 years of age, and 81% for patients over 16 years of age, and the 5-year graft versus host disease-free survival for patients under age 16 is 86%, and for patients over 16 is 77%.

So are there any downsides of undergoing an unrelated donor transplant? Well, yes. There is an increased risk of chronic graft versus host disease. And for some people, trading sickle cell disease for chronic graft versus host disease is really not a risk they're willing to take.

Announcer:

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