

Transcript Details

This is a transcript of an educational program. Details about the program and additional media formats for the program are accessible by visiting: <https://reachmd.com/programs/project-oncology/balancing-benefits-and-risks-in-emerging-sickle-cell-therapies/36236/>

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Balancing Benefits and Risks in Emerging Sickle Cell Therapies

Announcer:

This is *Project Oncology* on ReachMD. On this episode, we'll hear from Dr. Yogindra Persuad, a physician in the Department of Hematology at St. Jude Children's Research Hospital in Memphis, Tennessee. He'll be discussing the current therapeutic landscape for sickle cell anemia.

Here's Dr. Persuad now.

Dr. Persuad:

For a long time—since the discovery of sickle cell back in the early 1900s—there were really no treatment options for the disease. In the early 1990s, hydroxyurea was the first disease-modifying therapy for sickle cell disease and was primarily approved in adults who have severe sickle cell disease, so primarily the hemoglobin SS and S beta null types. Then, fast-forward to the early 2000s, that was approved for pediatric patients with sickle cell. Throughout all of this time, that was really the only disease-modifying therapy for sickle cell, and it has remained a cornerstone of sickle cell therapy.

Around 2016 was when we first got other disease-modifying therapies, and these were primarily used to address pain symptoms. There's not much approved right now for sickle cell. In patients who have acute illness or severe illnesses, sometimes we can use red blood cell transfusions.

In the last two to three years, that's when gene therapy for sickle cell has been approved, and we see, at least here in the United States, a push towards patients to get gene therapy. It doesn't cure them, but at least you can increase your fetal hemoglobin, and theoretically, that can decrease the complications surrounding sickle cell disease. And it seems to work, at least from the early clinical trials.

With hydroxyurea, it is oral, so you take it every day, the way you would like a vitamin. Some of the drawbacks are the capsules can come in different sizes, so they're large capsules that may be difficult for pediatric patients to swallow. What we see is, when patients take them, they respond, at least for the most part. But there seems to be a subset of patients that may be nonresponders. And then the other thing is we noticed that as patients get older and older, the response seems to wane over time.

And then the main drawback is patient compliance. We are left up to the patient to take the medicine, so if a patient is prescribed it but not taking it, you're not going to see the benefit of it, versus a curative therapy—it's thought to be a one-and-done option. And if you receive this curative option, whether it's the conventional bone marrow transplant or the transformative therapy, such as the gene therapy, you are ultimately alleviated from a lot of your symptoms.

But the problem with gene therapy and even on bone marrow transplant is in order to prep an individual for these therapies, you have to expose one to immunosuppressive therapy. And that can lead one to having a lot of infections and sores in the mouth. Some kids can have a type of syndrome called PRES, where you have elevated blood pressure and stroke-like symptoms while undergoing this. And then there is the risk of infertility and the risk of hair loss. In the bone marrow transplant, there's the risk of graft-versus-host disease, which may be a more debilitating disease than sickle cell itself. It can be disfiguring in the sense that you can have graft-versus-host of the skin. and then with all of them, there is the risk of secondary malignancies. And so these are the main drawbacks to the gene therapy or bone marrow transplant. But I would say the big success story is it can really alleviate the symptoms.

Announcer:

That was Dr. Yogindra Persuad talking about management strategies for sickle cell anemia. To access this and other episodes in our series, visit *Project Oncology* on ReachMD.com, where you can Be Part of the Knowledge. Thanks for listening!