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/advances-in-disease-modifying-and-curative-therapies-for-sickle-cell-disease/35928/

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Advances in Disease-Modifying and Curative Therapies for Sickle Cell Disease

Announcer:
This is *Project Oncology* on ReachMD. On this episode, we'll hear from Dr. Alexis Leonard, who will be discussing curative strategies for sickle cell disease. Dr. Leonard works in the Department of Hematology at St. Jude's Hospital in Memphis, Tennessee.

Here she is now.

Dr. Leonard:
When we think about curative strategies for sickle cell disease and how they fit, in general, to treating our patients with sickle cell disease, I generally think of treating sickle cell disease in two large categories. One is the category where the vast majority of patients will receive their care through things like disease-modifying therapies. So this is the patients that come to us in clinic routinely who we can offer strategies such as hydroxyurea or chronic blood transfusions—which we now have very good data and evidence for reducing complications related to the disease—or some of the newer therapies like crizanlizumab, glutamine, or voxelotor, until that was taken off the market last fall. So those are the types of treatments that we know have good evidence but simply do not correct or fix the complications of the disease. They help to minimize them for sure, but certainly, patients can go on and continue to have complications despite these disease-modifying therapies.

The alternative bucket or strategy are these curative strategies, where a narrow portion of patients will seek a curative option as compared to the vast majority who will receive standard disease-modifying therapies and supportive therapies for acute or chronic complications. So when we talk about the curative strategies bucket, for a long time we were only talking about allogeneic transplant, which we have been doing for nearly 40 years, so we have a lot of experience using allogeneic donors for our patients., Historically, this has always been best with a matched sibling donor, and there have been trials that have been done using matched unrelated donors and haploidentical donors, which certainly expands the donor pool, and what we've learned—you know, fast forward 30-some odd years—is that matched sibling donor transplants continue to be our optimized strategy. I would say, within the last five years, haploidentical transplants are approaching outcomes that we see with the matched sibling donor.

Now, alternatively, within the last 10 years, there's been a lot of focus on fitting gene therapy into that curative strategy landscape. We started the first trials for gene therapy about 10 years ago now, or a little over that, with the Bluebird Bio study. That study evolved over multiple cohorts to what it is now and really shaped the landscape for gene therapy for sickle cell disease. So, within the last five years, we've had a lot of trials and studies that are showing significant transformation and benefit to patients, but these studies are new and despite two of these therapies—the study from Bluebird Bio using lovo-cel and the study from Vertex using exa-cel—being FDA approved, we still have a lot more to learn and understand and improve upon in patients receiving gene therapy.

Announcer:
That was Dr. Alexis Leonard talking about how we can optimize sickle cell disease care with curative strategies. 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!