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Time needed to complete: 43m

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Cell and Gene Therapies for Sickle Cell Disease

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.

Emerging gene and cell therapies have the potential to greatly change the trajectory of sickle cell disease. So what is gene therapy? Well, gene therapy is a way to use one's own bone marrow stem cells to either cure their sickle cell disease or reduce their symptoms. And this is very different from a conventional transplant in which someone else's bone marrow stem cells are transplanted into a patient to give them new healthy cells that don't sickle.

Gene therapy is performed by removing circulating blood stem cells from the patient using an apheresis machine and a peripheral IV or central catheter. These circulating stem cells are then sent to a specialized laboratory where 1 of 2 different techniques are applied, either gene addition or gene editing. Now, what are those? So gene addition. So in gene addition, a new healthy copy of the gene that codes for hemoglobin A is added to the stem cells, and this allows the cells to begin to make hemoglobin that does not cause sickling. Now the second way to perform gene therapy for sickle cell disease is called gene editing. And there are two ways to perform gene editing. This is either through gene silencing or gene correction.

Right now there are two leading experimental gene therapy techniques that are closest to approval. The first is CRISPR-based technology, which involves editing a gene that suppresses fetal hemoglobin. This editing switches off this repressive gene, which then allows for the expression of fetal hemoglobin to occur. This replaces the sickle hemoglobin and reduces sickling. The second technology is LentiGlobin-based. This is a gene addition strategy that adds back a gene that codes for healthy hemoglobin A, and then allows the cells to make hemoglobin that does not cause sickling. To date, both strategies have demonstrated really promising results with significantly reduced anemia and significantly reduced vaso-occlusive pain episodes.

Now the logistical, the ethical, the cost benefit, and patient selection issues that are associated with these emergent gene therapy technology approaches are really complex and have not been completely worked out. As it is, sickle cell patients, particularly adults in our country, have really poor access to basic care. And it's really conceivable that access to more complex care, like gene therapy that requires significant expertise and infrastructure, might be even harder for these patients to access. It's also very likely that the cost of gene therapy will be north of $2 million.

Additionally, gene therapy does have some inherent risks that include infertility, and other chemotherapy-related risks like secondary bone marrow cancers. So it will be really critical for patients with sickle cell disease to be seen and counseled in centers of excellence in both gene therapy and sickle cell disease, so that the balance of these risks can really be properly assessed and individually tailored.

This discussion has focused on what promises to have great potential in the long-term management of sickle cell disease, possibly even a cure. Unfortunately, our time is up. Thanks for listening.

Announcer:
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