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Time needed to complete: 34m

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# Why Do We Need New Therapies for Sickle Cell Disease?

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**Dr. Freiermuth:**
This is CME on ReachMD, and I'm Caroline Freiermuth. Here with me today is Dr. Biree Andemariam. It is clear that we need to search for more and better treatment options for our patients with sickle cell disease, especially options that improve quality of life, reduce disease, and possibly even offer a cure. Dr. Andemariam, what are your thoughts on the rationale and developmental approaches being taken for sickle cell disease in drug research and in clinical trials?

**Dr. Andemariam:**
Thank you for your question. First, I'd like to discuss the advantages and drawbacks of the most used available treatments for sickle cell disease today. Now, first, I'll start with transfusions. They are really good for stroke prevention and for reducing the severity of the acute chest syndrome when patients present with that complication. However, the utility of transfusions in the prevention of pain crises and their use in pregnancy remain very not well defined. Transfusions are very time-consuming for patients, they take half to an entire day, for patients when they have them performed in the outpatient setting. And they're fully dependent on the availability of a compatible donor unit. It can be really difficult to find a peripheral vein in patients in order to insert an IV for transfusions. And oftentimes our patients require that the placement of an indwelling port-a-catheter. These catheters can get infected, and they can develop blood clots, and have to be removed and replaced. So they're not really, you know, very easy to manage. Patients with transfusion requirements often develop secondary iron overload and this requires them to take medications to chelate the iron.

The next thing that we use very commonly is hydroxyurea. We've had decades of experience with it. It was approved back in 1998 for its ability to reduce pain crises in individuals with sickle cell disease. I can tell you that it has excellent results if it’s started early in life, and its use is consistent and the dose is high enough, but the issue is that oftentimes, it's not dosed to its maximal tolerated dose. Oftentimes, it's not offered to patients, and there are a lot of patient concerns regarding its impact on fertility, the potential for teratogenicity, as well as the need for frequent blood monitoring for potential myelosuppression.

But there are newer disease-modifying therapies that are available, and for the most part, I have found that patients are keen to try them. Their use seems to be largely driven by disease manifestations and patient preference. And what I mean by, is that a drug like voxelotor, which is an anti-hemolytic agent, that is most commonly given to patients who have symptomatic anemia or a lot of red blood cell alloantibodies that makes the identification of matched blood very difficult. For patients who have frequent pain crises, for example, we can use L-glutamine or crizanlizumab. L-glutamine works by reducing the frequency of vaso-occlusive pain episodes. And crizanlizumab is an anti P-selectin monoclonal antibody that has the same clinical effect.

So where's the active research going on right now in sickle cell disease? Well, I think what we need to do is identify agents that can both improve anemia and reduce vaso-occlusive episodes. One agent that can do both would be outstanding. We also need to identify drugs that can stop a vaso-occlusive pain crisis once it has begun. We still don't have such a medication. And there's gene therapy in the

pipeline and on the horizon, which is a curative intent therapy that goes beyond conventional allogeneic stem cell transplantation.

**Dr. Freiermuth:**
Thank you so much for that overview, Dr. Andemariam. To follow up, where is most research focused, adult or pediatric? And once we discover new agents, where will they fit in our treatment algorithms?

**Dr. Andemariam:**
Good questions. Well, I think most of the research starts in adults. And as they're found to be efficacious and safe, what I see is that most of those trials then begin to expand into the younger age groups. And where will these new treatments fit? Well, I think it depends on whether or not they are disease-modifying therapies that have to be taken chronically, perhaps for the lifetime, or whether or not they have curative intent. And I think there's going to be a lot of patient preference with respect to that.

**Dr. Freiermuth:**
I would like to thank Dr. Biree Andemariam for joining me today. Unfortunately, our time is up. Thanks for listening.

**Announcer:**
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