Sickle Cell Disease chapter 3

**Sickle Cell Disease Series: How Atrium Health and its physicians are leading the field in trials and treatment**

Sickle cell disease can be debilitating and cause a wide variety of medical issues. Because Atrium Health is a leader in the sickle cell disease treatment, research and trials, we can provide our patients with alternative, new and advanced therapy to manage and treat the disease.

**Sickle Cell Treatment Options**

There are two FDA approved drugs for sickle cell treatment; hydroxyurea, which has been on the market for 20 years and is also commonly used in chemotherapy, and Endari, which was approved in July 2017. Endari is the first sickle cell treatment medication to be FDA approved in over two decades.

Daniel McMahon, MD, pediatric hematologist and director of pediatric sickle cell program often prescribes both medications for his patients. However, Endari isn’t FDA approved for patients under the age of 5 yet.

Endari works by injecting glutamine into the body to help reduce red blood cell clogs by making red blood cells less sticky thus able to flow easier. The new drug ultimately helps reduce patient pain crises, leading to less hospitalizations and fewer emergency room visits. Patients on Endari have also experienced fewer cases of acute chest syndrome, a common sickle cell occurrence according to the Federal Drug Administration.

Atrium Health’s Levine Cancer Institute was a principal site for Institutional Review Board approval for Endari with Ifeyinwa Osunkwo, MD, MPH, acting as the principal investigator. Dr. Ify, as she prefers to be called, is a hematology specialist with Atrium Health’s Levine Cancer Institute.

**Trials for Transplants**

Stem cell transplants, also known as bone marrow transplants, is an option for sickle cell patients that essentially replaces the patient’s unhealthy blood-forming cells with new, healthy blood-forming cells. A successful stem cell transplant can be a cure for sickle cell patients.

However, the transplant requires a matched relative donor, such as siblings who do not carry the sickle cell disease trait. Only 18 percent of eligible patients have perfectly matched donors, so more work is needed to be done to find matches or work-arounds for patients not perfectly matched with available stem cells.

In a new trial being conducted with Atrium Health, patients get Haploidentical stem cell transplants; which means a half-matched donor is sufficient for their transplant. This opens up the donor and recipient pool significantly.

Atrium Health’s Haplo trial is part of the Blood and Marrow Transplant Clinical Trial Network trial BMTCTN 1507, with Dr. Veeramreddy as the principal investigator. The trial qualifications are narrow for now, but the hope is that the study will open up the trial for more participants in the coming years.

**Pain Management**

Patients with sickle cell disease suffer chronic pain, prompting long-term use of pain medication. Often after patients have spent a lot of time in the hospital to address pain issues, their bodies may develop a slight to dramatic dependence on pain medications. Over time, this dependence on opioids means that patients continue to experience pain during a crisis because their tolerance is so high. Additionally, they are at risk to suffer withdrawal symptoms so severe, that it makes it nearly impossible for them to have a job, go to school, or just function as a healthy, independent adult.

One of the latest drugs being explored to manage sickle cell disease at Atrium Health is buprenorphine. While the drug has typically been used to treat opioid addiction, Atrium Health is offering it to sickle cell patients as an alternative to a steady pain medication regimen.

Dr. Ift is the first physician at Atrium Health to make buprenorphine a successful treatment option for sickle cell patients after tracking this pattern of dependence. She spent months getting trained on buprenorphine from Stephen Wyatt, MD, medical director of addiction medicine at Atrium Health, learning how it could benefit her patients.

Buprenorphine helps ease that withdrawal while also helping to treat the pain. So far, multiple patients at Atrium Health have been successfully treated with this medication.

“We have more than 55 patients that we've taken care of (with this treatment),” said Dr. Ify. “We’ve gotten them off of narcotics; they're out of the hospital, they're getting jobs, starting new careers. I mean, it's been amazing.”

While buprenorphine does not work for all sickle cell patients, it has helped enough to motivate Dr. Ify and her team to share their outcomes among the medical community.

“Atrium Health is leading the charge with this treatment,” said Padmaja Veeramreddy, MD, who works closely with Dr. Ify. “We are writing papers on our findings and other hospitals are in the beginning stages of looking at buprenorphine for sickle cell disease treatment, looking at Atrium Health as their guide.”

***Patient Spotlight***

*Sickle cell disease patient Evelyn Wilson was diagnosed with the disease at 18 years old, after finding she had the sickle cell trait at age 6. Her official diagnosis was prompted by crisis-level pain that landed her in the emergency room. That started a cycle of pain treatment in the emergency room, lasting as long as 21 days at a time in the hospital.*

*Over time, her body built up a tolerance to the pain medications. Stronger doses and more medications were needed to treat Wilson’s pain. After years of being seen by a hematologist, Wilson was referred to Dr. Ify to help her really deal with the sickle cell side-effects. After a few years of treatment, Dr. Ify recommended that they try buprenorphine for Wilson’s pain.*

*“The medication actually makes you feel up and lively, with no pain,” said Wilson. “So, I'll put it this way, I smile more. My family enjoys me more. This is really helping.”*

*Wilson takes the medication twice a day and hasn’t had pain crisis or been to the emergency room since being on the medication.*

Thanks to clinical research and clinical trials at Atrium Health, sickle cell disease management and treatment has come a long way in the last decade.