gp Greenwich-Post.com
THE NEWS OF GREENWICH, CONN. A HERSAM ACORN SITE

Fight rages on against devastation of Cooley's anemia

By Ken Borsuk on July 14, 2014

http://www.greenwich-post.com/27871/fight-rages-on-against-devastation-of-cooleys-anemia/

Peter Chieco, his daughters Jennie and Michelle and his wife Rose Ann have made fighting Cooley's anemia, which Michelle has, a mission that could soon pay off with big breakthroughs in treatment. Below, Mr. Chieco has long had the support of his employers at Morgan Stanley as he poses with Bradley Barber, complex manager for Morgan Stanley Wealth Management in Greenwich.

Not many people are aware of the devastating impact of a rare disorder known as Cooley's anemia, but for Peter Chieco and his family it's a daily reality. Cooley's anemia, which is also known as thalassemia, is a rare genetic blood disorder that impacts the body's ability to make red blood cells, leaving those with it severely anemic. Children born with it require lifelong blood transfusions, sometimes as often as every two weeks, plus daily drug treatments. Mr. Chieco, while physically fine, carries the trait for it with smaller than usual red blood cells. When a person has the trait there's a 20% chance of having a child who cannot make red blood cells, odds that unfortunately led to Mr. Chieco's daughter, Michelle, being diagnosed with it.

Known as an orphan disease because it impacts less than 10,000 people in the United States and 300,000 globally, Mr. Chieco said it needs the attention, since major breakthroughs could be made within the next 10 years with continued research, development and support.

“This is an extremely devastating disease because in order to alleviate the condition and treat it you have to get red blood cells from someone else,” Mr. Chieco said. “The disease really manifests itself within the first year of life, most times within the first six months. When a child is born they’re born with blood called fetal hemoglobin and that’s perfectly normal but it’s supposed to switch over to adult hemoglobin in the first year where you get your blood type. That’s where this disease hits. The switch is defective. A child suddenly becomes very anemic and pale in the first year. At that point, the only way to stay alive is to get someone else’s red blood cells that match your type.”

This has led to decades of involvement for Mr. Chieco, who works in Greenwich at Morgan Stanley as a financial adviser. He is the past president of the Cooley’s Anemia Foundation and is still actively involved with the medical advisory board. And that’s a lot of work because this is a disease that requires constant vigilance. The blood transfusions are only part of the treatment.

Mr. Chieco estimates that his daughter, who is now 25 years old, has gone through hundreds of thousands of milliliters of red blood cells that have been put in her to keep her alive. She’s been doing this since she was one and Mr. Chieco compares the process to having to have a gas tank filled. But while it keeps her alive, it also leads to other complications for her and others suffering from the disorder.

People with Cooley’s anemia might not be able to produce red blood cells but still have iron in their bodies at the normal level. So when they get the transfusion of the adult hemoglobin, that results in them having too much iron, which is extremely toxic as it accumulates in the body. In the past, the transfusions would help keep children alive but all the iron would kill them before they were 10 years old. In the 1980s there was a breakthrough when a drug called Desferal was released as an iron chelator.

Once you take the drug, it “grabs” the iron out of the bloodstream to excrete it from the body, removing the toxicity. That’s the good news. The bad news is that it’s not as though you can just pop a pill and be OK. In order for the drug to work it has to be delivered through a pump to be worn for 12 hours every night without exception.

“The iron is there constantly and you have to get rid of it,” Mr. Chieco said. “Ideally you would wear it 24 hours a day to get rid of all of it, but that’s very hard to do. My daughter and other children would basically go to sleep with it. It typically goes on the abdomen or a leg and it goes in like a thumbtack with a thin, plastic tube attached to a battery-operated pump… Doing this allows you to get the iron out and more blood in which allows you to live a more normal life.”

While this extends lifespans, it’s also extremely difficult to take, which Mr. Chieco said led to people not being as compliant as they should. Since iron overload can lead to heart failure, that just meant more deaths. But there have been steps in the right direction and within the last four years there have been two other iron chelators approved, one called Exjade, which is a drink, and one called L1, which is a pill. Mr. Chieco said this is stretching out lifespans even further because using all three iron chelators in different combinations gets the iron out more effectively.

“That’s the good news, but the really great news we’re hoping for lies in gene therapy,” Mr. Chieco said. “Even though there is treatment now for the disease, there’s no cure. You still have to have the transfusions. Things are better now, thank goodness, but our patient population was decimated by HIV and hepatitis before the blood system was fixed. It’s a constant worry when you’re getting blood. You’re hoping everything is safe and good, but it’s a risk.”

Mr. Chieco said there have been encouraging signs from tests in lab mice where genes are taken out and, essentially, genes that fight disease are given new instructions before being put back in. The hope is that in humans this can be injected into bone marrow to cause red blood cells to be made normally. Gene therapy could be the key for Cooley’s anemia and other single-cell disorders like sickle cell anemia, Tay-Sachs disease or even hemophilia.

That’s what the Cooley’s Anemia Foundation, which Mr. Chieco is such an active part of, is focusing on. Phase one human trials have started at Memorial Sloan Kettering Hospital in New York and companies are taking an interest. But safety is a critical concern, meaning the work is taking place slowly and carefully to ensure the treatment does no harm.

“If things go well we could really see something in the neighborhood of five to 10 years,” Mr. Chieco said. “This can be transformational. And gene therapy is just one thing being tried. There’s three or four other things coming up that have potential application to be transformational.”

As a financial adviser in Greenwich, where Morgan Stanley has been a big supporter of the foundation’s efforts, Mr. Chieco said he is seeing a big bounceback in the economy for biotechnology companies. As their stock prices go up, he explains, more funding comes in, which means more research and potentially more breakthroughs. Because of that he feels major progress in fighting Cooley’s anemia could be within sight as the research benefits from the inflow of capital.

“There’s money out there to really do studies,” Mr. Chieco said. “The stock market’s rebounds has really been a saving grace. It’s created wealth in the biotechnology area allowing people to invest.”

He adds that more money is needed, one of the areas the foundation works diligently at, but he has never been more optimistic that a transformative effect can happen in the coming years.

Mr. Chieco has three daughters, but Michelle is the only one with the disorder. He said she still uses the pump and goes in for the transfusions every two weeks, but, aside from that, Mr. Chieco says she looks like anyone her age would. It’s kept her from doing things, like sports, but she attended school normally at the Convent of the Sacred Heart in Greenwich and is now a pediatric nurse at NYU Hospital.

“She’s truly been one of the successes of the better treatments that have come along,” Mr. Chieco said. “The critical thing for all patients is to get the iron management right. It is lethal and it’s so difficult to manage it every day. The quality of life for many is the best it’s ever been, but not for everybody. Globally this is a real issue, especially in undeveloped foundations.”

More information is available online at Cooleysanemia.org.