Hospital's pioneering gene therapy aims to free patients of blood disease. Is a cure at hand?

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Born with a deadly blood disease, Clint and Alissa Finlayson's adopted daughters—Ada, nine, and Lily, 12—are the first patients on the West Coast to receive a new gene therapy offered by UCSF Benioff Children's Hospital Oakland.

Already, Ada is already feeling better 10 weeks after receiving her stem cell transplant. Lily started treatment last week. Both have 90% chance of a permanent cure.

"It's science, and it's a miracle," said their mother Alissa, sitting in the small yard of their guest home in downtown Oakland, far from their small mountain town of Kalispell, Montana.

The children were born with beta thalassemia, a common hereditary red blood cell diseases in China. Unable to create normal blood cells, they've needed six-hour-long blood transfusions every 21 days, an intense treatment that carries risk and requires constant monitoring.

Because Chinese orphanages can't provide treatment, both girls were destined to live short lives.

Then the family found that UCSF's Oakland hospital is one of three sites in the U.S. to offer the initial test of the therapy, Zynteglo. Now that Zynteglo is FDA approved, the hospital is among 15 in the nation authorized to provide care. Stanford's Lucile Packard Children's Hospital, Loma Linda University Children Hospital and Seattle Children's Hospital will also offer the treatment.

The therapy is a one-time treatment that works by using an engineered virus to deliver a healthy gene into patient cells. It's not the same as CRISPR, which uses gene editing to fix existing genes. That process is still under review and has not received FDA approval.

"The point of the treatment is to stop those transfusions," said Dr. Mark Walters, a hematologist and director of the hospital's Pediatric Blood and Marrow Transplant Program. Walters will follow the girls and other patients for 15 years to see if there are long-term complications, or if they remain free of disease and can be considered truly cured. Other patients are in the hospital's pipeline.

Beta thalassemia is caused by a single mutation on the gene for hemoglobin, the protein in red blood cells that carries oxygen to tissues. Children develop life-threatening anemia. They can't gain weight or grow properly. They suffer organ damage.

By fixing the underlying genetic problem, the new treatment buoys hopes for an estimated 1,300 to 1,500 patients—and opens up the possibility of treating other simple inherited disorders.

Scientists say this approach will be a crucial part of 21st century medicine. An estimated 400 million people worldwide are affected by one of the 7,000 diseases caused by mutations in a single gene.

"It's incredibly exciting time, as we harness what we've learn about genes and then how to fix them," said Walters.

"This is just one disease we're treating with the gene therapy," he said. "There are lots and lots of others to work on. All the lessons we've learned about genetics are coming to fruition."

These customized treatments remain challenging to build and are

profoundly expensive. Zynteglo, made by Massachusetts-based biotech company bluebird bio, costs $2.8 million for a single-use vial, making it one of the most expensive drugs in the world.

But money is saved by a lifetime without ongoing care, which can cost many millions of dollars.

Ada went first for the treatment. From start to finish, the process took four months.

First, her stem cells were collected from her blood. Using a virus, healthy copies of the hemaglobin gene were inserted into these collected cells, then grown for three months.

Chemotherapy killed off the bad stem cells in her bone marrow to make room for the new healthy cells. Her hair fell out. The cells were infused into her body, and found their home in her marrow. They are now beginning to pump out normal hemoglobin.

The beauty of this approach is that patients don't reject their own bone marrow. And there's no risk of a dangerous complication caused when foreign cells attack the body's own tissues.

For the first time in her life, Ada needs no transfusions. While her hemoglobin levels won't fully stabilize for a year, her spunk is emerging as the disease departs.

Lily's treatment, delayed by a cell manufacturing error, has now started. It was a major disappointment, because the girls could not be treated together. And instead of staying in Oakland for four months, the family must be here for eight.

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