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Researchers turn deadly virus into a life-saving cancer drug

By Addie Morfoot

In the spring of 2012, 6-year-old Emma Whitehead was close to death. She was suffering from acute lymphoblastic leukemia, a form of cancer.

After she was diagnosed in 2010, Emma's parents, Kari and Tom, originally tried a traditional cancer treatment - chemotherapy - to cure their daughter. But after each chemotherapy treatment, Emma's cancer reappeared.

Photo: Kari Whitehead

Desperate to save their only child, Kari and Tom decided to enroll Emma in an experimental treatment at the Children's Hospital of Philadelphia in April 2012. The experimental treatment involved collecting and removing millions of Emma's T-cells - a type of white blood cell that is a vital part of a human's immune system. Those T-cells were then infected with a disabled form of HIV, the virus that causes AIDS.

Serial Killer Cells

"The virus has been engineered so it can't cause disease anymore," Dr. Carl June of the University of Pennsylvania said in the short film "Fire With Fire". "But it still retains the ability to reprogram the immune system so that it will now attack cancer cells."

The **altered** T-cells are injected into the patient's veins where it is hoped that they will multiply.

"We call the modified immune (T) cells, serial killer cells," June told Ross Kauffman, who directed the film about Emma's treatment. "Each infused cell can kill more than 1,000 different tumor cells," June explained.

The experimental procedure made Emma very ill. She was placed on a ventilator to help her breathe and her body temperature reached 105 degrees. Swollen and unconscious, Emma's family prepared for her death.

"Emma had breathing difficulties and blood pressure difficulties," said Dr. Stephan Grupp of Children's Hospital of Philadelphia. "We knew that she could not have gotten any sicker without actually dying."

But Emma didn't die. The modified T-cells that had been inserted into her body eventually began to grow and started to fight the cancer in her system. On May 2, her 7th birthday, Emma woke up feeling better. Her fever had decreased and she was able to breathe on her own again. She was also cancer-free.

"It was like the calm after the storm," June said in the film. "The clouds went away and she woke up and there was no leukemia. When that child survived, it was an amazing event."

Treatment Does Not Work For All

While Emma was the first child to receive the experimental treatment, she was not the first patient in the advance stages of leukemia to undergo the procedure. A handful of adults have also tried to cure their cancer with the disabled HIV procedure.

According to an article in The New York Times, "three adults with **chronic** leukemia treated at the University of Pennsylvania have also had complete remissions, with no sign of the disease."

However, six other adults and one other child who received the treatment still have leukemia.

"The reality is the dramatic responses of cancer to new treatments are very unusual," Grupp told Kauffman. "We need to make it clear when we talk to a family that (this treatment) may not work."

According to The New York Times. "the research is still in its early stages, and many questions remain. The researchers are not entirely sure why the treatment works, or why it sometimes fails."

Researchers are also not sure if each patient who has gone into remission needs to keep the modified T-cells in their system forever. The altered cells live in a patient's bloodstream but in much smaller quantities. The modified T-cells not only destroy cancer cells, they also destroy some healthy cells, which means patients are at risk of becoming sick from infection.

"These T-cells are living drugs," Dr. Michel Sadelain of the Sloan-Kettering Institute told the newspaper. "With a pill, you take it, it's eliminated from your body and you have to take it again."

The mixed results of the treatments and the questions about its long-term side effects did not stop Novartis, a global drugmaker, from giving University of Pennsylvania researchers $20 million. The grant money will be used to further the development of the drug in the hope of eventually offering it to more leukemia patients.

A Cheaper Cure

Currently, bone-marrow transplants are the procedure for the majority of leukemia patients when all other options run out. Bone marrow is the soft, spongy, fatty tissue inside your bones. A bone-marrow transplant replaces damaged or destroyed bone marrow with healthy bone-marrow stem cells. The transplant is not only **grueling**, its results are uncertain. It is also a very expensive procedure – with costs estimated to be in the six figures.

Although using altered T-cells requires individualized engineering, the procedure is much more - `economical than having a bone-marrow transplant.

"Producing engineered T-cells costs about $20,000 per patient," Dr. June told The New York Times. "Scaling up the procedure should make it even less expensive."

While the economics are an important aspect of this experimental treatment, researchers and leukemia patients alike would like to find a cure for the disease - no matter what the cost.

Just ask Emma's parents. Last year their daughter was deathly ill and today she is in complete remission and leading a healthy life. Emma will enter third grade this fall.

Morfoot, A. Researchers turn deadly virus into a life-saving cancer drug. Newsela. May 19, 2013