FALL 2017

St. Baldrick's FOUNDATION
Conquer Childhood Cancers

HOW YOUR COMPANY MAKES AN

impact

Phineas Sandi was diagnosed with leukemia at age 4, but he didn't respond to standard treatments. His desperate parents turned to a St. Baldrick's-funded clinical trial testing a new immunotherapy to kill cancer. It saved his life.

Breakthrough!

Phineas was one of the first children in the world treated with CAR T cell immunotherapy. This groundbreaking treatment recently became one of the first two FDA-approved gene therapies in the United States. This "living drug" reprograms the immune cells of kids with acute lymphoblastic leukemia (ALL) to attack and kill cancer.

Phineas' parents, Carlos and Kristina Sandi, had already lost a baby girl, Althea, to incurable leukemia. Six years later, Phineas was diagnosed with ALL. Since ALL is considered the most treatable pediatric leukemia, they remained hopeful. Then, doctors told them Phineas wasn't responding to treatment. Kristina recalls, "I was ready to just lie down on the floor and dissolve."

Since Althea's death, Carlos had closely followed advances in pediatric leukemia research, including CAR T cell therapy. Together with Phineas' oncologist, he found a clinical trial at the National Cancer Institute in Bethesda, Md. A month later, St. Baldrick's Scholar Dr. Daniel W. "Trey" Lee began treatment. Dr. Lee cautioned Carlos and Kristina that preliminary results showed promise, but there were no guarantees. "Our response rate was pretty high, but we still didn't know the side effects,"

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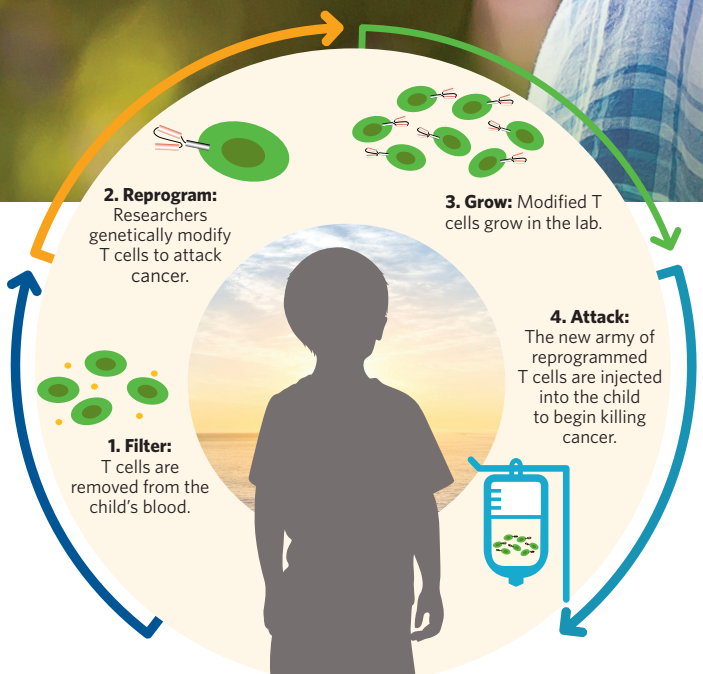

WARRIORS AT WORK

T cells are immune system warriors that attack and kill disease. But cancer cells are smart. Some, like those in patients with relapsed acute lymphoblastic leukemia (ALL), release proteins as force fields that prevent T cells from attacking. Now, St. Baldrick's-funded researchers have discovered a way to reprogram T cells to overcome those barriers and kill cancer. Stephan Grupp, M.D., Ph.D., the St. Baldrick's Foundation-Stand Up To Cancer Dream Team member who led another gene therapy clinical trial at Children's Hospital of Philadelphia, says, "It is the first FDA-approved gene therapy in the United States. This is a landmark moment for cancer research and for pediatric oncology."

Q&A Miracle Maker

St. Baldrick's Scholar Dr. David Barrett is one of the researchers who developed a revolutionary new immunotherapy to treat pediatric leukemia. Learn more about how he and the talented team at Children's Hospital of Philadelphia (CHOP) marshaled the power of kids' immune cells, called T cells, to kill cancer—and save hundreds of young lives.

Why did you choose pediatric cancer research?

I absolutely adore pediatrics because children inspire you in the simplest ways. They are so resilient. If they have to stay late for a treatment, they don't get upset. They say, "Great, I can stay here and play with Pat more." They have such an innocent approach to everything.

"St. Baldrick's donors fund ideas on cutting-edge, game-changing research that saves children's lives."

I learned very early on that I liked the concept of research, solving puzzles that were put before me. On a personal note, my father died of lung cancer when I was 19, and it had a profound influence on me. Cancer devastates families. It's a puzzle I want to solve: how can I, as a physician, make it less bad for children and their families?

Emily Whitehead

How did this clinical trial come about?

When I was a fellow at CHOP, I worked with a project on immunotherapy and leukemia in the lab of Dr. Stephan Grupp, a member of the St. Baldrick's Foundation-Stand Up To Cancer Dream Team. That project was a direct precursor to this breakthrough. At the time, T cells hadn't worked in actual patients, but the therapy showed promise in mice. In 2010, it was tried on the first three adult patients, and two of the three went into remission. It was the first time anyone had seen a disease response that dramatic. After that, we fought to open a clinical trial in children with relapsed acute lymphoblastic leukemia (ALL).

Tell us about Emily Whitehead.

Emily was the first child treated in our clinical trial, back in April 2012. She was only 6 when her ALL relapsed. At the time, we felt we had made a huge breakthrough that could work in children because it was successful in adults. This was the first time CAR T cell immunotherapy would be used in a child. It was exciting, but it was also terrifying. You don't know what you don't know. We knew that adults got pretty sick with flu-like symptoms as CAR T cells were killing their cancer. But after receiving her own CAR T cells, Emily got sick in a way we hadn't anticipated. She was dying and we didn't know how to stop it.

Brilliant work by a huge team saved her life. Everyone put in a round-the-clock effort to come up with a therapy. We identified a drug used in arthritis patients that suppresses the protein causing her severe reaction. At the time, it was good fortune that we were right. It was such an amazing example of teamwork that turned out to be the key that changed Emily's life. Today, she's been cancer free for more than five years.

The FDA fast-tracked the approval process.

They awarded us "breakthrough status," which led the way to an accelerated approval process. Without that designation, it's hard to say how long it would have taken. We presented data to the FDA on 63 children treated in the clinical trial, and it was unanimously recommended for approval. Things can happen relatively quickly when you have meaningful analysis about something that is truly transformative. This is one of the few cases you can point to when the FDA approved a treatment for children first, rather than adults.

Does CAR T cell therapy work for every child with ALL?

Unfortunately, no. It works for the vast majority of children, and in fact, gets 90% of children treated with CAR T cells into remission. As we've gotten better at understanding it, that number continues to grow. However, it does not last forever in some patients. Sometimes it's a few months, sometimes it's four years. We still have a lot to learn about why some patients are much more likely to have their leukemia return.

Tech Conquers Kids' Cancer

$1 million

Goal: by the end of 2018

Year 1:
1 Event
$20,434

+

Year 2:
4 Events
$435,250


David Barrett, M.D., Ph.D.

Can it treat other forms of pediatric cancer?
We think so. T cells may have different problems in patients with different cancers. Right now, we're trying to understand what makes the ideal CAR T cell. A lot of the time, we're dealing with unhealthy T cells because they come from sick kids. We need those cells to be healthy and frisky, so they last the life of the patient. Once we have a target for other types of cancer, I want to be ready with a really good T cell. We're looking to develop that game-changing effect for children with other types of cancer.

What keeps you motivated?
Dr. Grupp and I are both researchers, but we're also clinicians who take care of patients. Some still die. That's not good enough. When you see a child die, it drives you back to the lab. The real children and real faces keep you going.

That's where St. Baldrick's comes in. I was a St. Baldrick's Foundation Scholar before we knew this immunotherapy could work. True to the foundation's mission, they financed my work before there was a successful clinical trial. St. Baldrick's donors fund ideas on cutting-edge, game-changing research that saves children's lives.

David Barrett, M.D., Ph.D., is an assistant professor in the Division of Oncology, Bone Marrow Transplant Section, at the Children's Hospital of Philadelphia. He earned his M.D. and Ph.D. from Virginia Commonwealth University. He has worked in the lab of his mentor, Stephan Grupp, M.D., Ph.D., since 2007.

Breakthrough! (continued)

Dr. Lee explains. "Phineas' situation was dire. For parents, that has to be incredibly scary."

Still, the couple felt that the trial was Phineas' best hope. "We knew there were many unknowns and risks with CAR T, but we also realized how bad Phineas' remaining options were," Carlos says. "It was the best awful option we had."

After receiving his own modified immune cells, Phineas experienced the severe flu-like side effects doctors now recognize as expected. He also had minor neurological issues that disappeared within a week. Twenty-eight days later, Phineas was in remission.

Today, Dr. Lee is at the University of Virginia Children's Hospital, where St. Baldrick's funding is helping to bring this pioneering treatment—and hope—to more kids and their families. Meanwhile, the Sandi family advocates for pediatric cancer research at St. Baldrick's events and in Washington, D.C.

Now cancer-free for four years, Phineas still doesn't understand his historic role in advancing pediatric cancer research. That may come later, but for now he is more focused on being a typical 9-year-old kid. "St. Baldrick's-funded research not only saved our son's life, it gave him back his childhood," Kristina says, adding proudly, "He has defied all expectations."

The New Frontier


Shannon Maude, M.D., Ph.D.

CAR T cell therapy is the tip of the iceberg when it comes to using a patient's immune system to fight life-threatening pediatric cancers. Today, St. Baldrick's-funded researchers are delving deeper to unlock the secrets of immunotherapy to create lasting cures for cancers that defy treatment.

In her clinical trial at the Children's Hospital of Philadelphia, Shannon Maude, M.D., Ph.D., is studying why some children either don't respond or relapse after receiving CAR T cells. Dr. Maude was involved in the clinical trial that led to the FDA's approval of CAR T cell therapy last summer. "T cells are living things that can last in the body for months, or even years," explains the St. Baldrick's Foundation-Stand Up To Cancer Dream Team member and St. Baldrick's scholar. "Now, we're making slight modifications to reduce the chance of T cells being rejected and improve the length of time they last in patients."

So far, the results are promising—100% of kids who had never received CAR T cells before entered initial remission. Among children who had a poor response to a previous CAR T cell infusion, 60% responded.

While there's still a lot more work to be done, Dr. Maude is excited about its potential to help more kids to live cancer free. "These children motivate me every single day. What's remarkable is that we're seeing much more than small, incremental change. But while these are dramatic breakthroughs, it's not enough. Support from St. Baldrick's is so incredibly vital for us to continue our research."

Total Raised:

= $455,684

Your company can help us reach our goal!

Find out how on page 4.

#TechConquersKidsCancer

Technology professionals are at the center of a powerful movement to fight childhood cancer. Tech Conquers Kids' Cancer (TCKC) unites tech firms around the world under a single goal: to raise $1 million for St. Baldrick's by the end of 2018.

As part of this global effort, David Etue organized one of four TCKC events this year. Shaves That Save took place at the RSA conference in San Francisco, a gathering of 45,000 IT security professionals. "St. Baldrick's funds a lot of technology research initiatives, and that's an affinity we can all align with," says Etue, vice president of managed services with Rapid7, adding, "I've gotten to know kids and families impacted by cancer. They deserve better. I've met kids who ultimately lost their battle. Thankfully, I've also seen a bunch where the treatment has worked, but they still live with long-term side effects and fear of relapse."

Together, TCKC partners are leading the fight for kids with cancer. But we need your help. Find out how your company can make an impact by contacting Robyn Raphael, director of corporate relations, at Corporate.Partnerships@StBaldricks.org or (626) 792-8247, ext. 248.

Tech Conquers Kids' Cancer, SAP
$227,224

Shaves that Save, RSA Conference
$26,325

Tech Conquers Kids' Cancer, Microsoft Bay Area
$5,210

Bill & Joey's Excellent Head-Shaving Adventure—The Next Decade
$176,491

Let's Connect



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Thank you!

Thank you for supporting lifesaving childhood cancer research. Through your gifts, you are helping the St. Baldrick's Foundation fund the best childhood cancer research, no matter where it's being done. **With $27 million in grants funded last year, we are the largest private funder of pediatric cancer research grants. YOU made progress possible!**

Ways to Give

Kids with cancer need cures now, and it starts with you. Donate to help us fund the most promising childhood cancer research. Give once or monthly. Start today.
stbaldricks.org/impact

GuideStar GOLD

The St. Baldrick's Foundation is a volunteer-powered charity committed to funding the most promising research to find cures for childhood cancers and give survivors long and healthy lives.