ISSUE 27

St. Baldrick's FOUNDATION | CONQUER KIDS' CANCER

YOU MAKE AN impact

For **Julia Siegfried**, heroes wear lab coats. Their super power: Pioneering pediatric cancer breakthroughs. These dedicated researchers have developed a new immunotherapy that significantly improves the survival rate for kids with leukemia, like Julia.

Super Power

Six-year-old Julia has a super power of her own: Spreading happiness with her bright smile and infectious personality. "Julia is the mayor everywhere she goes," says her mother, Grace Siegfried. "Last summer, a woman found me online after meeting Julia at a store. Her husband was sick and she was having a really rough day. Julia made her feel better."

An energetic kindergarten student who loves ballet and basketball, Julia's appetite and energy level ground to a halt in January 2024, when she was a pre-schooler. Her symptoms soon worsened and Julia lost the ability to walk. Grace rushed her first to urgent care, and then Children's Hospital of Philadelphia (CHOP). Specialists quickly diagnosed Julia with acute lymphoblastic leukemia (ALL), the most common type of pediatric cancer.

The breakthrough has been hailed as one of the most significant advancements in pediatric oncology in decades.

As Julia began chemotherapy, Grace learned of a Children's Oncology Group (COG) clinical trial for ALL. Supported through the St. Baldrick's Foundation, the trial was studying the effectiveness of an immunotherapy called blinatumomab—referred to as blina, for short—that had been successful in treating adults. Julia was placed in the trial's control group, not immediately receiving the immunotherapy.

But several months later, a miracle happened: Preliminary data showed that this immunotherapy, combined with traditional chemotherapy, significantly increased the three-year disease-free survival rate for children with ALL, while reducing serious side effects. Overnight, therapy for kids like Julia changed.

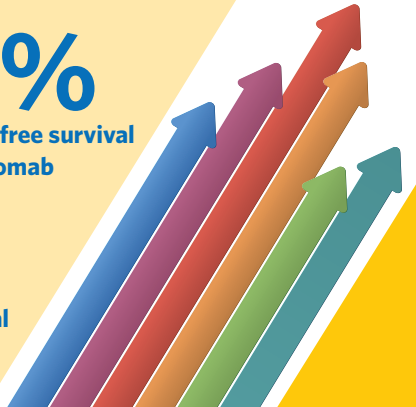
Continued on page 3

Miracle in the Making

There's new hope for kids with relapsed leukemia, thanks to a transformative immunotherapy pioneered by researchers with the Children's Oncology Group (COG). Called blinatumomab, it significantly raises survival rates for pediatric ALL, while reducing severe side effects. COG Chair Doug Hawkins, M.D., says, "For the first time, we can envision replacing toxic treatments with a safer, targeted therapy without compromising—and even improving—survival rates. We are grateful for the support of so many donors who helped make this discovery possible, including the St. Baldrick's Foundation."

96%
three-year disease-free survival rate after blinatumomab

87.9%
three-year disease-free survival rate before blinatumomab



Home Run!

Pediatric cancer researcher Rachel Rau, M.D., and a team of dedicated scientists recently pioneered a breakthrough in the fight against ALL—the most common type of childhood cancer. Their work has significantly boosted the survival rate for the highest risk kids.


Rachel Elizabeth Rau, M.D.

Your work has transformed care.

This is the most exciting thing that has happened in my career. We set out to determine if an existing targeted immunotherapy called blinatumomab—also referred to as blina—could improve outcomes for children with newly-diagnosed ALL (acute lymphoblastic leukemia). We opened the trial in 2019 through the Children's Oncology Group (COG), eventually enrolling 4,200 children at 215 centers in the United States, Canada, Australia, and New Zealand. While overall, children with ALL have a good chance of cure, we have not made any significant improvements in outcomes for over 20 years. It was time to try something new and investigational to try to move the needle.

In this randomized trial, the three-year disease-free survival (DFS) rate was 87.9 percent for those who got standard treatment alone. But for those randomized to get blina in combination with standard chemotherapy the DFS rate jumped to 96 percent, with minimal side effects. Put another way, there was a 61 percent reduction in relapsed ALL. Our findings were amazing: Blina isn't just a winner, it's a home run!

How did doctors respond?

The results of this clinical trial literally changed the standard of care overnight and galvanized pediatric cancer researchers. Last July, we released a memo with the primary results, and the very next day treatment sites across the nation began to offer blina to most children with ALL. Normally, a new treatment has to go through intensive FDA approval. Somewhat fortuitously, the FDA had granted an expanded indication for blinatumomab for newly-diagnosed ALL patients in June. That opened the door to make blina available to kids immediately without further FDA approval.

How does blinatumomab work?

Blina has been around for at least 15 years, and was initially approved by the FDA to treat adults with ALL. It's an immunotherapy that marshals a patient's T-cells to fight cancer. Traditional chemotherapies attack the DNA of cancer cells, or starve them until they die. But along the way, chemo can also damage normal cells, causing devastating side effects. Blina is a two-part molecule: One part binds to the patient's cancerous B-cells, and the other binds to the T-cells that fight illness. When brought together, the T-cells kill malignant B-cells, with minimal side effects.

Tell us about your team.

My co-chair, Sumit Gupta, M.D., Ph.D., from The Hospital for Sick Children in Toronto, and I are so grateful to all the investigators who worked on this trial—physicians, statisticians, nurses, pharmacists, and so many others. Blina is a fantastic agent, but it needs to be administered continuously via IV for 28 days straight. It's challenging to administer to an energetic 3-year-old. Our pharmacy and nursing colleagues have been amazing—they developed a specialized backpack that securely holds the IV bag, with a line that's less likely to be disconnected when a little one is rolling on the floor with a sibling. Our success was a total team effort.

What's next for you?

I'm motivated to keep working for kids with cancer. I grew up on an Ohio dairy farm in a very close-knit, rural area. My cousin, who was like a pesky little sister to me, was diagnosed with acute myeloid leukemia and unfortunately did not survive. Now, I want to do whatever I can to prevent that from happening to other kids and families. And every day, I get to see the results of my work—kids at the hospital who are now receiving blina. Back in Ohio, my mom is a Girl Scout leader. One of the girls in her troop has ALL and is being treated with blina; I was fortunate to speak with her mother, who was so excited. It's really cool to know that I helped to make that happen.

Members of the AALL1731 study team (L to R: Dr. Elizabeth Raetz, Dr. John Kairalla, Dr. Mignon Loh, Dr. Stephen Hunger, Dr. David Teachey, Dr. Sumit Gupta, and Dr. Rachel Rau) meet to celebrate the presentation of the results at the American Society of Hematology meeting in San Diego.

Now, the work continues for other kids. We're already designing new trials to see if we can use blina to reduce the amount of chemotherapy needed to treat ALL. That, in turn, could reduce the devastating side effects some kids experience.

"The results of this clinical trial literally changed the standard of care overnight and galvanized pediatric cancer researchers."

What's your message to St. Baldrick's donors?
The support of St. Baldrick's donors directly funded this clinical trial—you made this happen! Your generosity also continues to encourage young investigators to enter the field. My very first grant was from St. Baldrick's; without that grant, I'm not sure I would have pursued research as a career. Breakthroughs like blinatumomab can take years to develop. Just think—your gift today can fund the beginning of another promising clinical trial. Five or 10 years from now, your donor dollars will again achieve something great.

Rachel Rau, M.D., is co-chair of AALL1731, the groundbreaking Phase III clinical trial that established blinatumomab as a standard of care for children with acute lymphoblastic leukemia. A board-certified pediatric hematologist-oncologist at Seattle Children's Hospital, she earned a medical degree from The Ohio State University College of Medicine and completed her residency and fellowship at Johns Hopkins Children's Center.

Super Power (continued)

Grace Siegfried and her daughter, Julia.

The breakthrough has been hailed as one of the most significant advancements in pediatric oncology in decades. "I read about it online and first thought, 'Wow, I wish Julia wasn't in the control group,'" recalls Grace. "But a couple days later at the clinic, doctors told me that Julia was cleared to start blinatumomab."

Blina is administered continuously for 28 days through an IV in two cycles spread several months apart between chemotherapy treatments. Kids sometimes have flulike symptoms within the first day, so they spend a night in the hospital. Then, they wear a backpack containing the IV solution for the next four weeks.

Despite the great news, Julia still had a lot of hurdles to overcome. She tolerated the immunotherapy well at first and quickly adjusted to carrying a backpack 24/7. But in the fall, Julia's second round of treatment was postponed when a prior infection flared up, colonizing her IV line. She completed blina in late November and is now on maintenance treatment to prevent the cancer from returning.

"Donations to St. Baldrick's are so impactful and needed now more than ever. I feel very fortunate Julia had a type of cancer that is very well studied and researched. Now, there are other kids with cancer who need our help."

Like many kids who receive chemotherapy, Julia is dealing with side effects including cognitive issues and extreme neuropathy requiring leg braces and physical therapy. But that doesn't stop this personable dynamo—she's looking forward to dancing on stage in her ballet recital in June, and 10 months later, ringing the bell to signal the end of her cancer treatment.

Grace is grateful to the St. Baldrick's Foundation donors who supported the COG clinical trial of blinatumomab, as well as other forms of pediatric cancer research. She and Julia are doing their part, too: The mother-daughter duo sell flower bouquets to spread joy and raise money for foundations like St. Baldrick's. "Julia and I want to get the word out about how important this is," Grace says. "Donations to St. Baldrick's are so impactful and needed now more than ever. I feel very fortunate Julia had a type of cancer that is very well studied and researched. Yet, there are other kids with AML (acute myeloid leukemia), neuroblastoma, and other types of childhood cancer who need our help."

Legacy of Hope

Richard and Alice Bucher with their granddaughter, Arden Quinn

The St. Baldrick's Foundation is pleased to announce the introduction of the Richard and Alice Bucher Emerald Circle, a new giving opportunity for grandparents and others who designate a planned gift to the foundation. The circle is named in honor of Alice and the late Richard Bucher, who lost their precious granddaughter, Arden Quinn Bucher, to childhood cancer in 2008. After Arden's passing the couple—along with the 3-year-old's parents, Amy and Rick Bucher—raised more than $1 million for pediatric cancer research. Before passing away last August, Richard named St. Baldrick's as the beneficiary of an IRA, establishing the Richard and Alice Bucher Emerald Circle.

"Through this bequest, Richard and I wanted to honor Arden and inspire others to support the St. Baldrick's Foundation," Alice said. "Someday—long after we're gone—there will be a cure for pediatric cancer. That's the legacy Richard and I wanted to leave."

At the heart of planned giving is your desire to create a meaningful legacy. "Through a legacy gift, donors can turn heartbreak into hope for kids with cancer," said Susan Heard, senior director of distinguished giving at St. Baldrick's. "We're making great progress, but the fight will continue for many, many years. Your legacy gift can be a beacon of hope, now and long into the future."

Establishing a legacy gift to St. Baldrick's is easy: Simply scan the QR code to get started or contact Susan Heard: Susan@stbaldricks.org.
To create a will designating St. Baldrick's as a beneficiary, go to https://www.freewill.com/stbaldricks.

Meet Our Legacy Donors

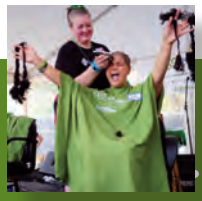
"Right now, my grandson, Sal, is benefiting from the groundbreaking blinatumomab trial featured in this newsletter. Research means survival for little guys like Sal—that's why I'm setting up a legacy gift to the St. Baldrick's Foundation."

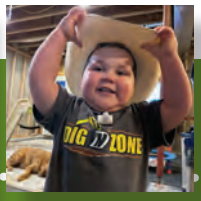
—Hartwell Dew

"Tim is a St. Baldrick's founder. We've met many families dealing with the devastating effects of childhood cancer. In their honor, we are leaving a legacy of hope through a provision in our estate plan to support St. Baldrick's. We are honored to invest in St. Baldrick's every year, but know that there will still be work to do when we are no longer here. This mission matters and our investment is critical."

—Tim & Sheila Kenny

Let's Connect





Stbaldricks.org/distinguished-giving **Stbaldricks.org/get-involved/business** **Stbaldricks.org/league**

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 $356 million in research grants in the U.S. funded since 2005, we are the largest charity 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 fund the most promising childhood cancer research. Give once or monthly. Start today: **stbaldricks.org/ways-to-give**

Platinum Transparency 2024
Candid.

Scan here to help find a cure!

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