



impact

Jeremiah "L.J." Graham is a typical teenager, thanks to the St. Baldrick's Foundation – Stand Up to Cancer Pediatric Cancer Dream Team. Pioneering immunotherapy saved his life. Now, the Dream Team is poised to create more miracles for kids with cancer.

Living the Dream

At 13, the shy seventh grader doesn't talk much about his four-year fight against leukemia. Diagnosed at age 5, L.J. battled cancer three times. A grim prognosis in 2014 led to his status as "Patient 48" in a clinical trial for a groundbreaking therapy, now approved by the FDA, that uses kids' own disease-fighting T-cells to kill cancer.

His mom, Jennifer, still cries when she recalls the moment she learned the treatment was successful. She and her husband, Jeremiah, credit the Dream Team and St. Baldrick's Foundation donors. "The researchers on the Dream Team were brave enough to envision and develop this treatment," the St. Louis mom says. "And donors were amazing enough to fund this. L.J. is happy and healthy. There's no better gift anyone could have given us."

Earlier this year, the St. Baldrick's Foundation announced \$16 million in additional funding over four years for the Dream Team, 150 researchers across nine institutions in North America. St. Baldrick's will invest \$8 million, with the member institutions contributing another \$8 million, through 2022.

That's great news to the Dream Team's co-heads, Crystal Mackall, M.D., and John Maris, M.D. Six years ago, the pair envisioned assembling an elite team of researchers from across the country. Their focus: the intersection between genetics and immunology. Specifically, they're looking at what triggers different pediatric cancers—and then developing targeted, disease-specific treatments.

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REACH FOR THE STARS

Diagnosed in 2014 with a brain tumor, Julia Alexander powered through treatment and is now in maintenance. This year, the 12-year-old from Virginia took on a new battle—lobbying on Capitol Hill with other St. Baldrick's ambassadors for passage of the STAR (Childhood Cancer Survivorship Treatment Access and Research) Act. Signed into law in June, the legislation increases funding for pediatric cancer research and provides support for survivors, who face a lifetime of health challenges stemming from treatment. Julia has lost six young friends to cancer. Her mom, Melissa, says, "Cancer is a battle we fight every day. We need all the money we can get to treat and cure this disease."



Q & A Giant Step

Dr. John Maris has discovered a protein that could pinpoint the genetic cause of neuroblastoma, the most common cancer in infants. Thanks to his determination—and new funding from the St. Baldrick's - Stand Up to Cancer Pediatric Cancer Dream Team—we're one step closer to a cure.

What drew you to neuroblastoma research?

When I finished college, I got a job in the lab at the University of Pennsylvania. As a brand new technician, I worked with two leading researchers from the Children's Hospital of Philadelphia (CHOP) to investigate why neuroblastoma behaves so aggressively in some patients, but very benignly in

"Our work with GPC2 is, by far, the most potent and exciting therapy that I've seen emerge in my career."

others. I got to meet patients and their families, and work alongside two very inspiring leaders in the field of childhood cancer. That early experience drove my decision to become a pediatric oncologist.

The ironic thing about these solid tumors is that the younger you are, the better your prognosis, so most babies with neuroblastoma are cured. The older patients—meaning 2- or 3-year-olds—tend to develop the more aggressive form of the disease. I've spent my career trying to find out why.

How has the field moved forward?

People used to look under a microscope and see neuroblastoma, or breast cancer or colon cancer. But what we now know is that there are clear genetic subsets within these cancers. We're better able to individually identify the type of neuroblastoma a patient has. That tells us what type of therapy might be effective in treating it. One of the big problems is that a cure is not enough, because many current neuroblastoma survivors have lifelong side effects of their chemotherapy and radiation therapy. So it's really important to be more precise in how we're approaching neuroblastoma and other diseases.

Progress can be slow. What keeps you motivated?

As a clinician, the patients I care for are the reason I do what I do. They're very inspiring, but some experiences can also be incredibly bittersweet. One

of the kids I treated for a decade died recently at 17. He was a really smart kid, who was impacted by experimental therapies and really understood the power of research. He and I had a long talk about how he could impact other kids by donating tumors from his body to our lab. Now, we're studying his cells to find out why the immunotherapies we tried ultimately failed him. It's very sad, but he's also provided us with incredible opportunities to help other kids.

Tell us about your latest research.

Through the work of Dr. Kristopher Bosse, a St. Baldrick's-funded Young Investigator grant recipient, we've identified a protein called GPC2 that is on neuroblastoma and other pediatric cancer cells, but not on normal tissues. We designed an antibody that goes in and seeks out the protein, and we have attached to it a very potent poison. The idea is that the cells expressing GPC2 will take in the antibody. The normal tissues will not, and therefore, they're not exposed to the toxin. So far, we've proven that a single dose is able to eradicate very aggressive neuroblastomas in mice, without affecting normal cells. Now, we're working with two pharmaceutical companies to develop this drug and test it in the clinic. It's very encouraging.

That's exciting news.

Our work with GPC2 is, by far, the most potent and exciting therapy that I've seen emerge in my career. We're all hands on deck to try to get this into the clinic. But at the same time, we know that any drug in development could fail at multiple different steps, and therefore, we never put all our eggs in one basket. It's important to have a diversified portfolio. So while we're very excited about how promising this looks, we're also working on CAR-T cell immunotherapy as a backup strategy.

What's the Dream Team's role?

The basic concept for the Dream Team is to use the power of genetics and genomics to discover the right immunotherapy approaches. Previously, these two fields were largely siloed from each other, yet they were two of the most productive fields in



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INCREASED DONOR GIFTS

\$300,000 PER YEAR

FOUNDATION MATCH

\$300,000 PER YEAR



Dr. John Maris

childhood cancer research. Now, we have a great group of individuals across North America who have a coordinated research effort that is trying to impact all of the high risk cancers. We've made an incredible amount of progress over the five years that St. Baldrick's has been funding us.

Clearly, St. Baldrick's donors make a difference.

St. Baldrick's and Stand Up to Cancer were enthusiastic about investing more money in our program because they saw the pace of the research and the number of patients we're impacting. Over the life of the funding, we've treated over 700 children on pediatric cancer immunotherapy trials, many of whom benefited tremendously. I can tell you for sure that a large part of that would not have happened without the funding. The lives of so many patients and their families have been improved because of our program. That's something that should make donors very proud.

John M. Maris, M.D., is a pediatric oncologist at Children's Hospital of Philadelphia and co-head of the St. Baldrick's - Stand Up to Cancer Pediatric Cancer Dream Team. A clinician and scientist, he is an expert in molecular genetics who specializes in neuroblastoma. Dr. Maris is a graduate of the University of Pennsylvania School of Medicine.

Living the Dream (continued)

The team has made great strides with relapsed acute lymphoblastic leukemia (ALL), the kind L.J. had. With the new funding, they're now setting their sights on solid tumors. "We're very excited about what has happened with leukemia," explains Dr. Mackall, endowed professor of pediatrics and medicine at Stanford University. "But we're most excited about the CAR-T cell work in solid tumors we're doing in our lab right now."

While pediatric cancer research tends to move slowly, joining forces across disciplines has quickened the pace, with the potential to bring promising therapies to clinical trials faster. In his lab at Children's Hospital of Philadelphia, Dr. Maris has pinpointed a protein he believes may be a trigger for solid tumors and other types of pediatric cancers (see page 2).

Both researchers say funding is key to advancing their efforts. "A lot of things can come together and move quickly when you have adequate resources," Dr. Mackall adds.

Today, L.J. is more intent on fishing, playing soccer and video games than thinking about leukemia. But once in a while, he and Jennifer chat about the battle that almost claimed his life. "L.J. knows that what he went through is kind of amazing," Jennifer says. "People will be learning about Patient 48 for years to come. I tell him, 'Doctors are developing treatments now for so many more kinds of pediatric cancer because of you.'"



Dr. Crystal Mackall

**Childhood Cancer Awareness Month:
3 Ways to Help Kids With Cancer**

September is Childhood Cancer Awareness Month. At the St. Baldrick's Foundation this month, we're shining a brighter spotlight on kids battling cancer and the researchers who are working to find cures. Here are 3 ways you can help:

1. Learn more about pediatric cancer on our website: StBaldricks.org
2. Increase your gift. The chart below demonstrates the tremendous impact we can make if just 600 people donate an extra \$500 to pediatric cancer research through the St. Baldrick's Foundation.
3. Recruit a friend to match your donation, join you if you participate as a shavee, or host an event in your community or business.



per year:

3 = \$1.8 million

YEARS

MORE TO FIGHT PEDIATRIC CANCER





Lifesaving Legacy

Through a legacy gift to the St. Baldrick's Foundation, you can impact kids with cancer for generations to come. We're extremely grateful to the founding members of The Emerald Circle, who have chosen to include St. Baldrick's in their estate plans:

Michelle Renaldo Ferguson

Tom & Susan Heard
Estate of Hilding Johnson
Joseph Lucchese
Gloria Marino
Bette C. Moates Trust
Peggy McDaniel
Kathleen Ruddy
Michael Ryan
Karen & Stu Slifkin
Michael Tucker
Marian Ward Charitable Remainder Unitrust
Marvin Weinberg Revocable Trust
Anonymous (8)

To be recognized as a member of The Emerald Circle, please provide the foundation with a copy of your will or estate plan. Contact Kym Hoffman, strategic director, foundation and legacy giving: Kym.Hoffman@StBaldricks.org or 626.792.8247, ext. 242.

Giving Runs in the Family

"St. Baldrick's is changing the rules of fighting childhood cancer, giving children hope at beating cancer and a healthier life."

Michelle Renaldo Ferguson first shaved on April 14, 2012. Her cousin Tom's wife, Susan Heard, had lost her son, David, to cancer. Susan took her tragedy and made it her mission to help other families battling cancer. "Susan is the ultimate salesperson," Michelle said. "She set my financial goal at \$5,000. And every time I hit my goal, she raised it."



Michelle was afraid she was going to cry when she shaved. "I placed my youngest nephew in front of me, thinking he would see silly Aunt Michelle shaving her head and laugh, and that would make me laugh, too. There was something remarkably cathartic about it. It was a life-changing moment. I raised just under \$25,000 and the event raised a total of \$82,000."

Michelle said her grandparents had instilled in her family a sense of duty, and giving to St. Baldrick's contributes to that ethos. "David galvanized all of us. Susan organized an event

in which my brother and his partner walked from Franklin Lakes, NJ, to Bethlehem, PA — over 100 miles. We all grew up in different states and at different times, but we all want to remain close. Giving to St. Baldrick's supports that sense of family."

Michelle, who lives in New York City and is Chief Operating Officer at publishers ABRAMS, NYC, knew that she couldn't continue to shave, so she thought about alternative ways to give. That's when Planned Giving and Estate Planning came to mind, and now she is a member of The Emerald Circle.

"St. Baldrick's is changing the rules of fighting childhood cancer, giving children hope at beating cancer and a healthier life," Michelle said. "I'm just grateful I have the ability to give back to such a worthy organization doing such great work."



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 over \$253 million in grants funded since 2005, 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



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.