

WINTER 2016 IMPACT REPORT

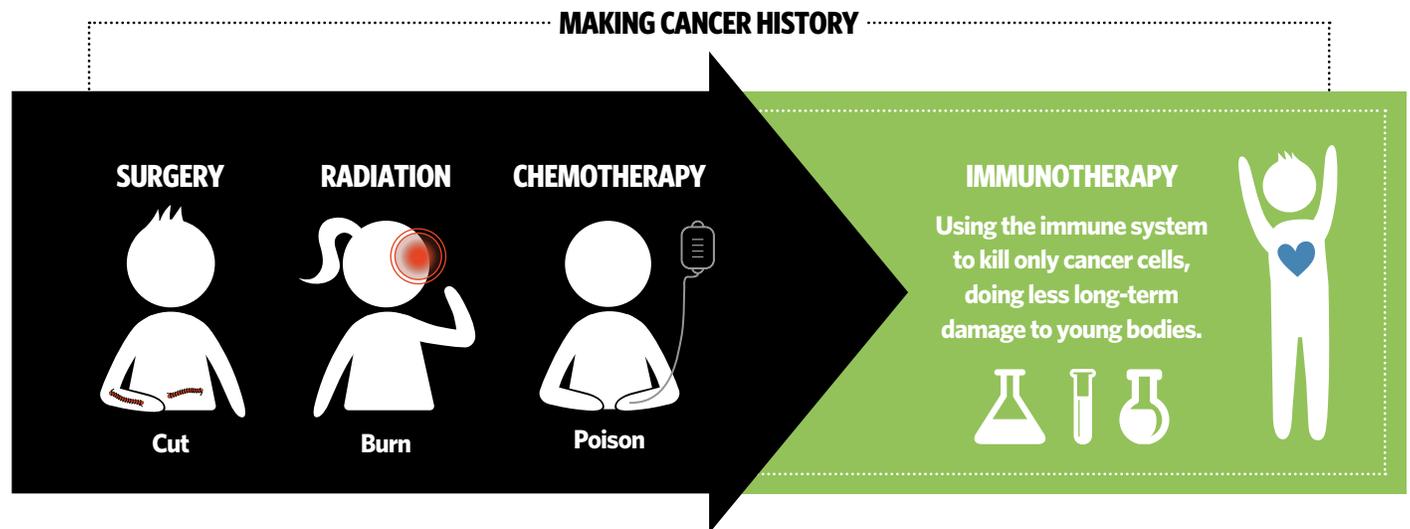
With St. Baldrick's laser focus on research, we want you to know how **your support** is making a difference for kids with cancer.

What's new?

St. Baldrick's researchers are at the forefront of an entirely new kind of treatment. The concept is simple: *Put the immune system to work*, fighting off cancer just like it fights off the common cold.

Researchers are finding ways to modify the immune system so it can identify, target and kill cancer cells, leaving healthy cells unharmed.

Since children first began to survive cancer in the 1950s, they've endured surgery, radiation and chemotherapy – to cut out, burn or poison cancer cells. But these treatments also damage healthy cells, and more than 80% of survivors suffer long-term chronic illness as a result.



Immunotherapy

Here are just three of the immunotherapy research projects currently funded by St. Baldrick's:

- While most kids with B cell acute lymphoblastic leukemia (ALL) are cured with standard chemotherapy, for those who relapse, the outlook is grim. To change that, Dr. Kevin Curran at Memorial Sloan Kettering Cancer Center in New York is working on gene therapy to create “cancer assassins” — anti-CD19 CAR modified T cells, to be exact.



Dr. Curran notes, “*St. Baldrick’s has supported the use of CAR T cells to fight leukemia since the inception of this therapy. Without that support, the success we see today in the most difficult leukemia cases would not be possible.*” Dr. Curran and his colleagues are now attempting to develop CAR T cells that treat solid tumors, as well. He believes that with your support, we can provide new treatment options for children with cancer — “*treatment that only targets the cancer cells, without the long-term side effects we see with chemotherapy and radiation.*”



Paul Quinn, owner of Jim Brady’s, proudly stands next to the honoree board.

Dr. Curran often speaks at the St. Baldrick’s event on St. Patrick’s Day at Jim Brady’s Restaurant & Bar, site of the original St. Baldrick’s event. The pub was selected as the National Philanthropy Day 2015 Outstanding Philanthropic Small Business for the New York Chapter of the Association of Fundraising Professionals. Dr. Curran is the *AVM Traders St. Baldrick’s Scholar*, in recognition of a company that has raised more than \$1 million for research with St. Baldrick’s.

- Imagine the devastating effects of surgery, radiation and chemotherapy on a young child with medulloblastoma, the most common pediatric brain tumor — hearing loss, cognitive deficits and more.

At the University of Alabama at Birmingham, Dr. Gregory Friedman’s research uses the herpes virus to defeat medulloblastoma. With one gene removed, the virus can’t replicate and can’t kill healthy cells — but it *does* kill tumor cells and even the cells that can make the tumor grow back. The virus also stimulates the immune system, which could give this therapy a one-two punch toward knocking out medulloblastoma.



This treatment has proven to be safe and effective for adults, and Dr. Friedman’s laboratory research is moving toward a clinical trial for kids fighting medulloblastoma.



Dr. Friedman’s Scholar award is funded with generous support from the Miracles for Michael Fund, a St. Baldrick’s Hero Fund created in memory of Michael Orbany, honoring the boy’s tremendous strength and relentless spirit. To learn more about creating a Hero Fund to honor a loved one, contact **Kelly** at 888-899-2253 or Funds@StBaldricks.org.

- Great news is already coming out of the *Stand Up to Cancer – St. Baldrick’s Pediatric Cancer Dream Team* project. The first of its kind, this \$14.5 million four-year grant sets out to cure many of the most hard-to-treat childhood cancers.

Dream Team researchers from seven institutions across North America are working together to find new ways to use genomics (the study of genes and their functions) and immunotherapy to target cancer cells. Most kids with acute lymphoblastic leukemia go into remission after only a few rounds of chemo, but about 20% of them will relapse, drastically reducing their chance for a cure.



Phineas, celebrating his cancer victory as a 2016 St. Baldrick's Ambassador.

"Every day with our son is a gift. Without the Dream Team immunotherapy trial, we would have lost Phineas." —Tina, Phineas' mom

About 1% of kids with ALL never get into remission despite intense treatment. For them, there is little hope. Phineas was one of those kids. His parents had already lost one child to a different type of leukemia and they knew all too well what this meant for their son.

With no good options left, Phineas was enrolled as the 10th and youngest patient in a Dream Team clinical trial at the National Cancer Institute, testing a new form of immunotherapy on kids with relapsed and otherwise untreatable leukemia.



Amazingly, 93% of patients enrolled in Dream Team trials showed no evidence of disease after just one infusion of modified T cells. One year later, 55% of those patients remained in remission.

PARTNER WITH THE BEST

When other foundations want to partner with a leader to fund the most exciting research, often the first place they come is to the St. Baldrick's Foundation. This was true for Stand Up to Cancer (SU2C) — a program of the Entertainment Industry Foundation — when it set out to fund a pediatric cancer Dream Team. If you are interested in partnering with St. Baldrick's to fund the very best childhood cancer research, please contact **Susan at 888-899-2253 or Partnerships@StBaldricks.org**.

The team is now discovering why some patients relapse and how to prevent that in the future.

As for Phineas, because of this clinical trial — and his doctor, St. Baldrick's Scholar Dr. Daniel "Trey" Lee — Phineas is cancer free, and has been for two years.

Survivorship

There is much to celebrate when a child survives cancer — but also much to worry about. One study shows that by the time they are 45, more than 95% of survivors will have a chronic health problem and 80% will have severe or life-threatening conditions, as a result of the treatments they received for childhood cancer.

Of the many conditions survivors face, the two most dangerous are heart disease and secondary cancers.

- Congestive heart failure is not a concern for most people in their 20s or 30s. But for the many childhood cancer patients treated with chemotherapy drugs called anthracyclines, heart failure can begin within just 5–10 years of treatment. One in 10 of those patients will develop the condition, and half of those will die within five years.

A study made possible by St. Baldrick's donors, opening at dozens of sites around the U.S., could revolutionize care for these patients. Led by Dr. Saro Armenian at the City of Hope in Duarte, California, the research consortium is testing whether low doses of a drug called carvedilol may protect the hearts of young cancer survivors.



"The potential gained from this, in terms of really transforming the lives of survivors, is tremendous."
 Saro Armenian, M.D.

The St. Baldrick's grant funded the manufacture of the drug and the placebo, the computer network where patient data is recorded, and the randomization process so the researchers don't know which patient was given carvedilol and which patient was given a placebo, to avoid any bias.

If successful, this study will help avert a life-threatening complication for many childhood cancer survivors, without compromising their overall quality of life.



- Dr. Greg Aune was diagnosed with Hodgkin lymphoma at the age of 16. After surgery, chemo and radiation, he became a childhood cancer survivor. Because of that experience, he went to medical school and became a researcher.

At age 35, Dr. Aune had another life-changing event: emergency heart surgery to repair the consequences of the treatments he'd had as a teen.

With funding from St. Baldrick's, he now studies the long-term effects of chemotherapy on the heart, while treating childhood cancer patients and survivors at the South Texas Pediatric Cancer Survivorship Program in San Antonio.

"I can think of no other organization that pursues their goals with such passion and tenacity. . .and for all the right reasons. This organization is making a real impact every day. . .I truly believe that the St. Baldrick's Foundation will change the face of childhood cancer in this country and across the globe."

Gregory J. Aune, M.D., Ph.D., St. Baldrick's Scholar

Dr. Aune's involvement in St. Baldrick's stretches even further. He serves as a volunteer on the Foundation's Advocacy Committee, to help improve federal legislation and regulations affecting childhood cancer patients and survivors. He also not only shaves his head, but helps organize an annual St. Baldrick's event in San Antonio, galvanizing others to support the cause.



Thank You



None of this lifesaving research would be possible without people like you. Thank you for your steadfast support.

Questions? Please contact: Impact@StBaldricks.org