



Alex's Lemonade Stand Foundation
Beckwith-Wiedemann Syndrome
Impact Report





Alex's Lemonade Stand Foundation (ALSF) emerged from the front yard lemonade stand of 4-year-old Alexandra “Alex” Scott, who was fighting cancer and wanted to raise money to find cures for all children with cancer. By the time Alex passed away at the age of 8, she had raised \$1 million. Since then, the Foundation bearing her name has evolved into a worldwide fundraising movement and the largest independent childhood cancer charity in the U.S. ALSF is a leader in funding pediatric cancer research projects across the globe and providing programs to families affected by childhood cancer. For more information, visit AlexsLemonade.org.



With Gratitude

Dear Friend,

All of us here at ALSF would like to sincerely thank you for your support of Alex's mission to find new treatments and cures for childhood cancers including Wilms tumor and hepatoblastoma.

Your support is helping researchers to develop preliminary data, publish their findings, and push forward innovative treatment options. Thanks to you, we are closer to a day where no child with Beckwith-Wiedemann Syndrome (BWS) and related disorders will have to suffer from cancer.

We are truly honored to fight childhood cancer by your side. Thank you for being the driving force behind lifesaving cures. Please don't hesitate to reach out if you need anything from us here at ALSF.

Until there are cures,



Liz & Jay Scott

Alex's Parents & Co-Executive Directors

Alex's Lemonade Stand Foundation



Thanks to Supporters Like You

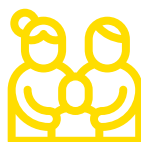
ALSF is the largest independent childhood cancer charity in the U.S., focused on funding critical research and supporting childhood cancer families.



More than \$300M raised since 2005



Funded over 1,500 medical research grants at nearly 150 institutions



Supported nearly 30,000 families through key programs like Travel For Care

ALSF is the only childhood cancer research organization that has been given the NCI Peer-Reviewed Funder Designation for rigorous selection of research and grants.

Meet a **BWS Hero**

JACOB



Jacob was born with Beckwith-Wiedemann Syndrome. Because of this, he visited the oncologist every six weeks for cancer screenings and check-ups, blood draws, and ultrasounds to watch for Wilms tumor and hepatoblastoma.

Jacob loves doing all he can to raise awareness for his syndrome and childhood cancer. Even today, he is always smiling and giving hugs, even to complete strangers. He has no fear and if any music begins to play within range he stops to dance his heart out! He has been such a trooper, even after having macroglossia and needing tongue-reduction surgery. His love for life fills the room and he brings a smile to everyone who is around him.

Like every parent, Jacob's parents want to watch Jacob grow up healthy, happy, and strong. They hate seeing him go through all the struggles life has thrown at him, and hope that he will never lose his amazing smile.

Jacob's mom, Jennifer, advises others facing BWS to acknowledge that the journey will be difficult, but always remember to smile and laugh through the storms, take pictures to keep the memories alive both of sad and happy times, and reach out to others for support. "One day, you will be able to provide help and support to another family going through the same struggles," Jennifer says. "And at that moment, you will think back and know that maybe some good can come from a multitude of heartaches."

ALSF is important to Jacob's family because of its support of researchers, who are dedicating their time to study Beckwith-Wiedemann Syndrome. Jacob's parents hope that, by sharing his story, they can encourage another family to know hope, and show them that there is a life beyond cancer.



Dr. Jennifer Kalish [right]

Finding Childhood Cancer Before It Develops

Dr. Jennifer Kalish of Children’s Hospital of Philadelphia has been awarded several grants from Alex’s Lemonade Stand Foundation, including a Research Catalyst Grant — an invitation-only grant, designed to fund gaps and advance the pace of innovative pediatric cancer research.

Most recently, Dr. Kalish co-authored a paper published in the scientific journal “Genes”. In this collaborative study, Dr. Kalish performed a large epidemiological investigation into the (epi)genotype, phenotype and population characteristics that could inform personal cancer risk within the BWSp population. They reclassified BWS from a syndrome to a spectrum (BWSp) given the heterogeneity of individual phenotypes.

Their results suggest that while some phenotypes may be associated with tumor development, the heterogeneity of the BWSp population (both clinical and molecular) presents a challenge to understanding the full spectrum of BWSp. They advocate for reconsideration of universal tumor screening for BWSp within other countries and healthcare systems.

BWS affects approximately one in 10,000 kids and is linked to increased risk of certain childhood cancers, including Wilms tumor and hepatoblastoma. With proper treatment and monitoring, these cancers are treatable, which makes it critical to properly identify kids with BWS.

2023 R Accelerated Award

Synthetic Gene Expression Regulator Switches (SynGERS) expressed in CAR T Cells to Cure Children with Solid Tumors



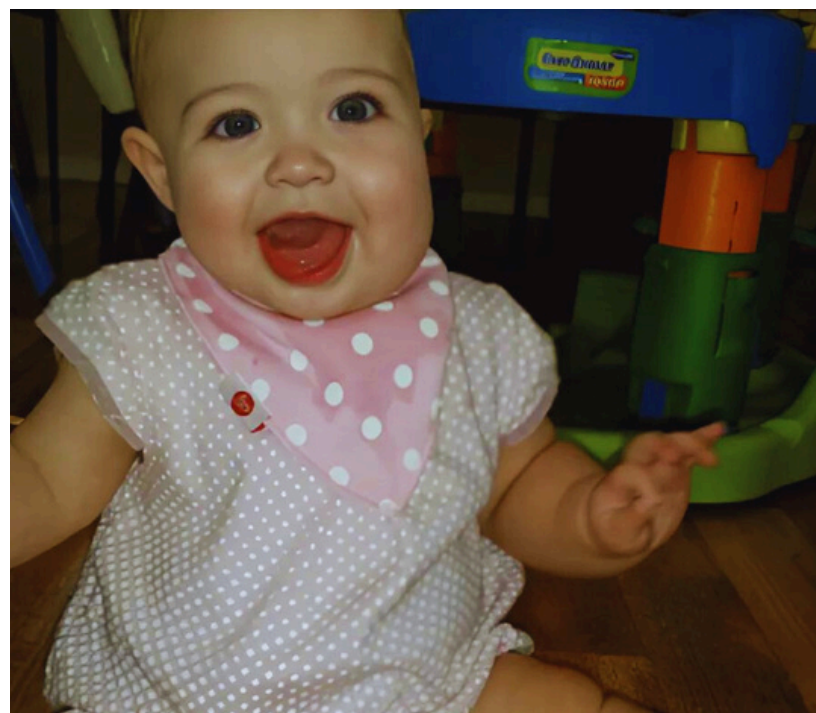
Andras Heczey, MD, Baylor College of Medicine

The immune system can recognize and eliminate cancer. Genetically engineering a unique type of immune cells called T cells can result in up to 90% complete elimination of leukemia cells. Such strategy holds exceptional promise for children with solid tumors like Wilms Tumor, a tumor associated with BWS. However, additional genetic engineering modifications are required to overcome the solid tumor microenvironment which can stop the engineered T cells.

Dr. Heczey's team developed a novel system called "Synthetic Gene Expression Regulator Switches" – SynGERS. There are several genes that can promote T cells' ability to survive and expand; and similarly, there are several genes that can block their antitumor function. These master regulator genes can be manipulated individually and have been shown to enhance the antitumor activity of engineered T cells. It is unknown which master regulator induces the most potent antitumor effect; furthermore, it is unclear what are the most effective combinations. To address this knowledge gap, Dr. Heczey will evaluate a library of SynGERS built to modulate the expression of master regulators of T cell function in a tumor-specific manner.



R Accelerated Awards encourage established investigators to push their research forward toward childhood cancer breakthroughs and ultimately new clinical interventions.



Thank You

for all you do to help kids with cancer!

