



# Alex's Lemonade Stand Foundation

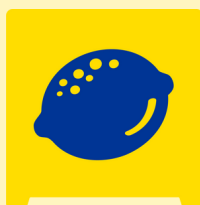
## Acute Lymphoblastic Leukemia

### 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. Her spirit and determination inspired others to support her cause, and when she passed away at the age of 8, she had raised \$1 million. Since then, the Foundation bearing her name has evolved into a national fundraising movement and is one of the leading funders of pediatric cancer research in the U.S. and Canada.



## 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 like acute lymphoblastic leukemia (ALL).

Your support is helping researchers develop preliminary data, publish their findings and push forward innovative treatment options. Thanks to you, we are closer to a day where no child will have to suffer from ALL.

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's a cure,



**Liz & Jay Scott**

Alex's Parents & Co-Executive Directors

*Alex's Lemonade Stand Foundation*



**Thanks**

to Supporters Like You

**122**

**ALL projects (and counting) have been funded**

“

The ALSF support is 'keeping the lights on' in the lab and making us competitive for future NIH funding."

— Dr. Craig Mullen,  
University of Rochester



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Understanding fundamental processes is critical to developing new targeted therapies. We greatly appreciate ALSF's investment in myself, my research, and my vision."

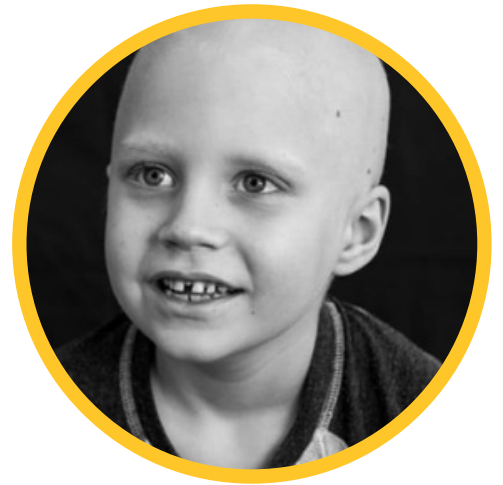
— Dr. Genevieve Kendall,  
Nationwide Children's Hospital

# Meet an **ALL Hero**

**Name: Beaudin**

**Favorites: Baseball and spending time with his dog Hatchet**

**Fun Fact: Beaudin is an avid speed-cuber**



In 2018, Beaudin was 6 years old fighting what seemed like a bad cold. Weeks of fevers and medical tests left his family with growing concern. Because most of his tests came back normal, it wasn't until 2019 that Beaudin received an official diagnosis: acute lymphoblastic leukemia.

The next 20 months revolved around Beaudin's treatment. His treatment regimen was straightforward, and he was fortunate enough not to have any prolonged hospital stays, adverse reactions or secondary conditions. As he moved towards the maintenance stage in 2020, the end of treatment seemed near, until a routine lumbar puncture revealed abnormal white blood cells in his spinal fluid.

Beaudin's mom came across a clinical trial at Children's Hospital of Philadelphia. The only problem – they would have to travel to Pennsylvania regularly from their home in Denver, Colorado. “We didn't know how we would cover all the costs, but when you are making these decisions – the life and death of your child – finances fall very low on the list,” she says. Fortunately, a social worker referred them to ALSF.

With the help of the ALSF Travel For Care program, the cost of airfare for Beaudin's family was covered so he could continue receiving the treatment he needed. “We are so thankful for the Travel For Care program for allowing our family the possibility of pursuing a relapse treatment option for Beaudin that we believe will be his forever cure. Having to make care choices for your child under the worry of financial pressure is something that no family should have to do.”

Today, Beaudin is more than two years cancer-free and living his best life!

# Research Spotlight: New Projects in ALL

## Elucidating the Roles of Aneuploidy in Down Syndrome-Associated Leukemia

**Chun-Chin Chen, PhD, Boston Children's Hospital**

Children with Down syndrome have an approximately 20-fold increase in the risk of overall leukemia, particularly acute lymphoblastic leukemia.

These children also have lower tolerance to chemotherapies due to complications, creating a complex situation for treatments of leukemia.

Dr. Chun-Chin Chen is using his newly awarded Young Investigator Grant to understand the mechanisms that make children with Down syndrome highly vulnerable to leukemia development. He is using a range of advanced genomic and genetic approaches to study Down syndrome-associated leukemia both in tissue culture and in animal models. He envisions that understanding these mechanisms will enable the discovery of more effective therapeutic options that can lower the doses of chemotherapies and reduce treatment-related complications in children with Down syndrome-associated leukemia.



## Novel Therapy for Pediatric Leukemia Patients with NUP98 Translocations

**Jolanta Grembecka, PhD, University of Michigan**

Dr. Jolanta Grembecka of the University of Michigan is now in the second year of her Reach Grant and is assessing rationally designed combinations of her menin inhibitor with selected FDA-approved targeted drugs used in pre-clinical models of the NUP98-rearranged leukemia. The goal of her research is

to assess the efficacy and mechanism of action of these combinations to provide a rationale for clinical translation of best combinations to the pediatric leukemia patients with NUP98 translocations. Based on data she's collected so far, she expects that at least some of the proposed combinations will outperform the effect of a menin inhibitor as a single agent, resulting in complete, long-lasting remission in vivo in the advanced pre-clinical models of the NUP98-rearranged leukemia, including patient-derived xenograft (PDX) models. Since the menin inhibitor is currently in clinical trials in AML patients with MLL1 translocations or NPM1 mutations, the outcome of this work should lead immediately to new clinical trials of the most promising combinations in pediatric leukemia with NUP98 rearrangements. In the long-term, this work should result in new therapies for aggressive pediatric leukemias like ALL with translocations of the NUP98 gene, which currently suffer from very poor prognosis.



## Where Are They Now?

### **Alix Seif, MD/MPH, 2008 Young Investigator Grantee**

ALSF Alum Dr. Alix Seif, an attending physician at the Children’s Hospital of Philadelphia, is two decades into her career as a leading childhood leukemia researcher, but what really helped her career take off is the ALSF Young Investigator Grant she was awarded in 2008. Following a different route than most researchers, Dr. Seif transitioned from the lab to epidemiology, which studies how diseases occur in different groups of people and why.

“Alex's Lemonade Stand Foundation (ALSF) was my first grant ever. And that was a game changer for my personal trajectory. It was just really exciting somebody recognized the value in what we were doing,” said Dr. Seif.

Dr. Seif’s first grant investigated a therapy that stimulates the immune system, much like an infection would. It was targeted for children with acute lymphoblastic leukemia (ALL). Using the theories of immunotherapy, Dr. Seif aimed to recruit the body’s own immune system to identify and kill cancer cells. When a child relapses with ALL, they usually do not respond to chemotherapy, requiring new treatment methods. Dr. Seif’s project offered a new option, hopefully creating a treatment for kids with high-risk leukemia that was both more effective and less toxic than chemo or a bone marrow transplant. It worked well in the lab and eventually led to early clinical trials. For Dr. Seif though, she saw an opportunity to study this effect on a grander scale.

That led to her epidemiology work, where she could study large data sets of kids with leukemia who developed serious infections, which activated the immune system similarly to how her therapy functioned in the lab, to understand the effectiveness of her approach.



**Thank You**

for all you do to help kids with cancer!

