2023

Alex's Lemonade Stand Foundation

Acute Myeloid Leukemia Impact Report

Alex's Lemonade Stand Foundation for Childhood Cancer™

AlexsLemonade.org

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 myeloid leukemia (AML).

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 AML.

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

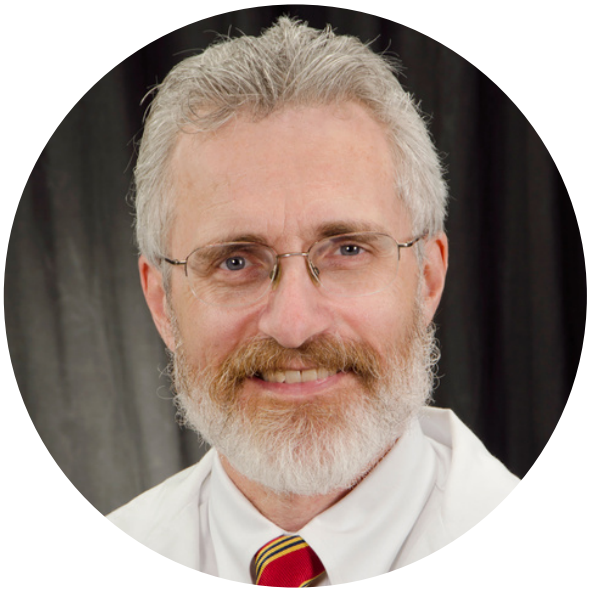
ALSF

Thanks to Supporters Like You

90 **AML 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



"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

ALSF

Meet an **AML Hero**

Name: Diego
Loves: Reading, writing stories for comic books, and building LEGOs
Favorite Movies: Star Wars and Batman

In early 2021, Diego started looking unusually pale. Then low energy, a fever, and strange bruises on his leg led him to the ER. He immediately began receiving blood and platelet transfusions at the hospital, then had a bone marrow biopsy and lumbar puncture exam. Diego was diagnosed with acute myeloid leukemia.

Treatment began with five rounds of chemotherapy at a nearby hospital in Las Vegas, during which Diego happened to get every side effect imaginable. His second round of chemotherapy caused his bone marrow to shut down, which meant he couldn't heal easily from infections. He needed a transplant, but getting his family and his donor to the hospital in California was challenging.

That's when his family's social worker connected them with ALSF. ALSF's Travel For Care program covered lodging and travel expenses for Diego's family, including his 6-year-old stepbrother who was going to be his transplant donor.

Finally, Diego received the transplant, and today he is happily in remission!

"I will be forever thankful for the help and relief that ALSF provided for our family! We weren't able to afford so much, and you were able to make this possible! Your support was invaluable since we were still trying to get our life together!" shared Alma, Diego's mom

Research Spotlight: New Projects in AML

Identifying Therapeutic Vulnerabilities in Pediatric AML through Investigation of the MECOM Transcriptional Network

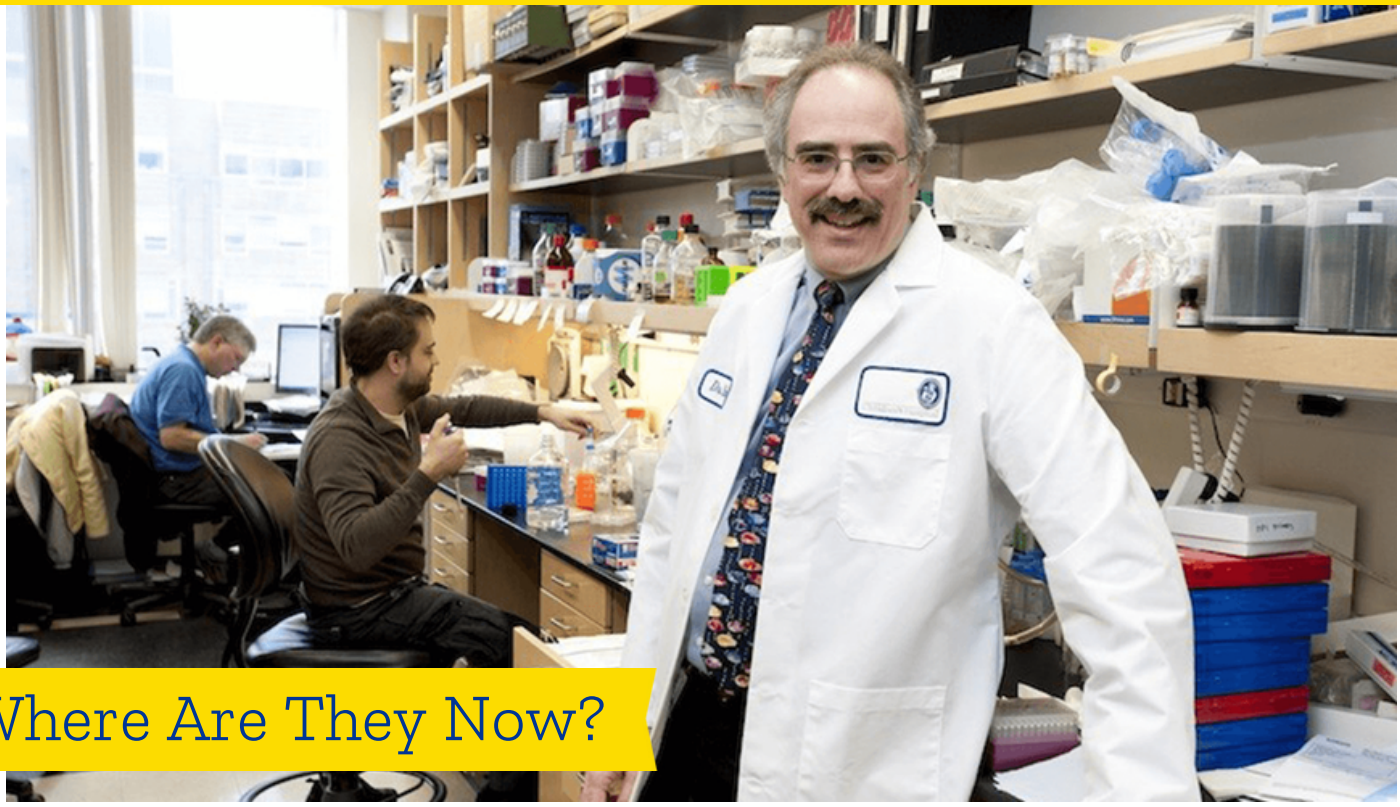
Richard Voit, MD/PhD, Boston Children's Hospital

Dr. Richard Voit of Boston Children's Hospital is using his newly awarded Young Investigator Grant to uncover the specific role of MECOM, a critical regulator of normal blood stem cells, in controlling an important gene network that makes a subset of AMLs very difficult to cure and to identify other proteins that could be easier to manipulate with treatments. To do so, he is using a cutting-edge strategy to engineer MECOM-addicted AML cells with a MECOM kill-switch that will allow him to specifically destroy the MECOM protein in a matter of minutes. This will allow him to uncover the immediate, specific effects of MECOM loss on the structure of DNA loops and expression of other genes. His prior work suggested that two proteins, RUNX1 and CTCF, become differentially activated upon MECOM loss and this strategy will allow the investigation of these interactions.

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.



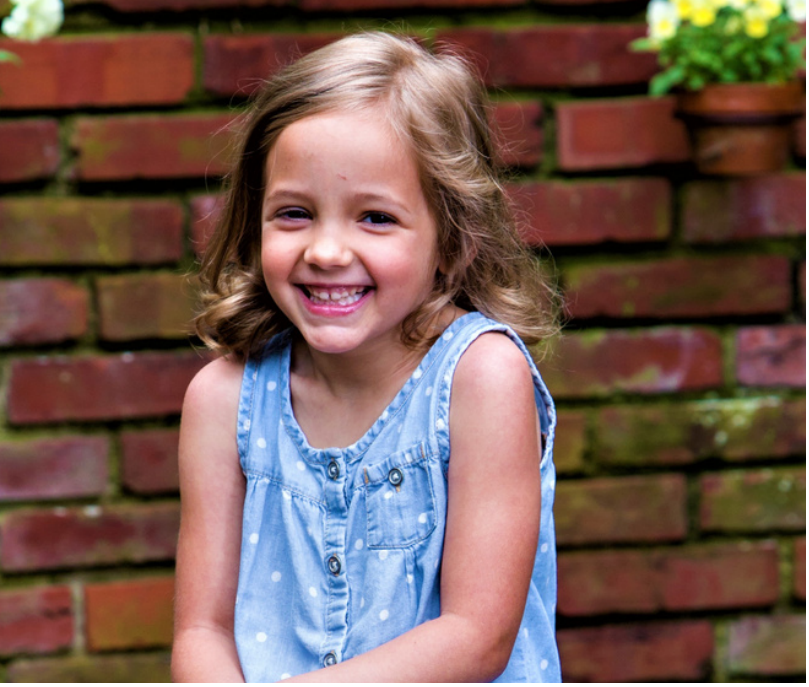
Where Are They Now?

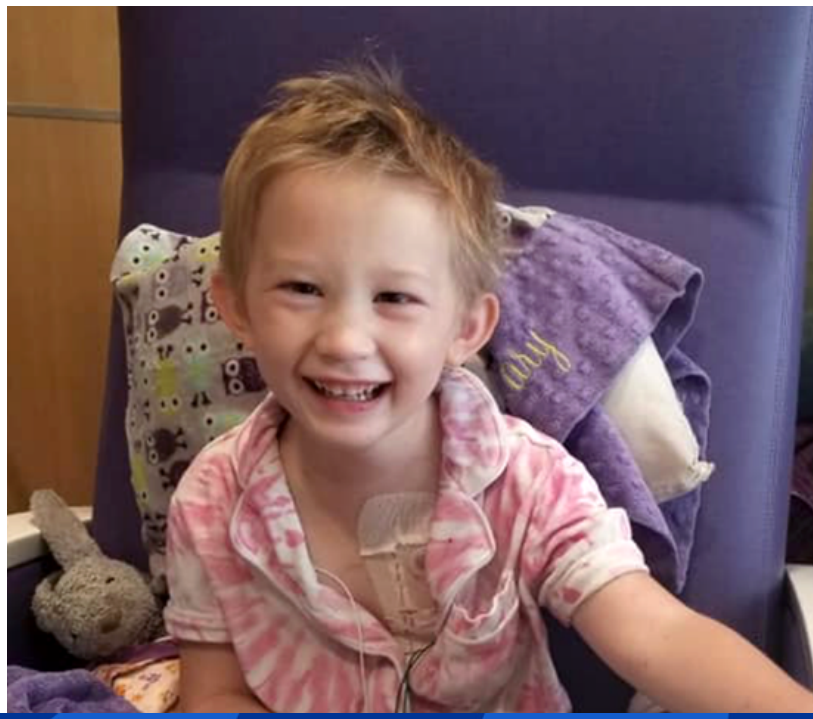
Leonard Zon, MD, 2016 Familial RUNX1 Research Grantee

Dr. Leonard Zon of Boston Children's Hospital got his start as an ALSF awardee in 2016 to model the process of leukemia initiation that is accompanied with expansion of an abnormal stem cell clone in zebrafish to better understand how the disease is established and how to treat it. Dr. Zon and his team used a special technique of coloring blood stem cell clones and their progeny. This resulted in groups of colored cells, each representing cells originating from a same-colored stem cell clone.

Since then, Dr. Zon's career has taken off and in 2021, he was awarded a multi-million dollar grant in ALSF's most competitive category: Crazy 8. Dr. Leonard and his multi-disciplinary team spanning six institutions across the U.S. and Europe have developed technologies to genetically fingerprint cells using a variety of techniques across multiple animal models. These methods will uncover when and where pediatric leukemias develop and how their origins impact disease course. Named the Breakthrough of the Year in Science Magazine in 2018, the zebrafish approach is being utilized again in Dr. Zon's Crazy 8-funded project to color each stem cell differently and track developments.

Dr. Zon also developed CRISPR-based fingerprinting approaches that offer unprecedented resolution into the ancestry of leukemic cells. These approaches have never been applied to cancer. Dr. Zon and his team are currently exploring these cellular fingerprinting techniques in normal blood development to understand how normal fetal stem cells contribute to blood production in adulthood.





Thank You

for all you do to help kids with cancer!