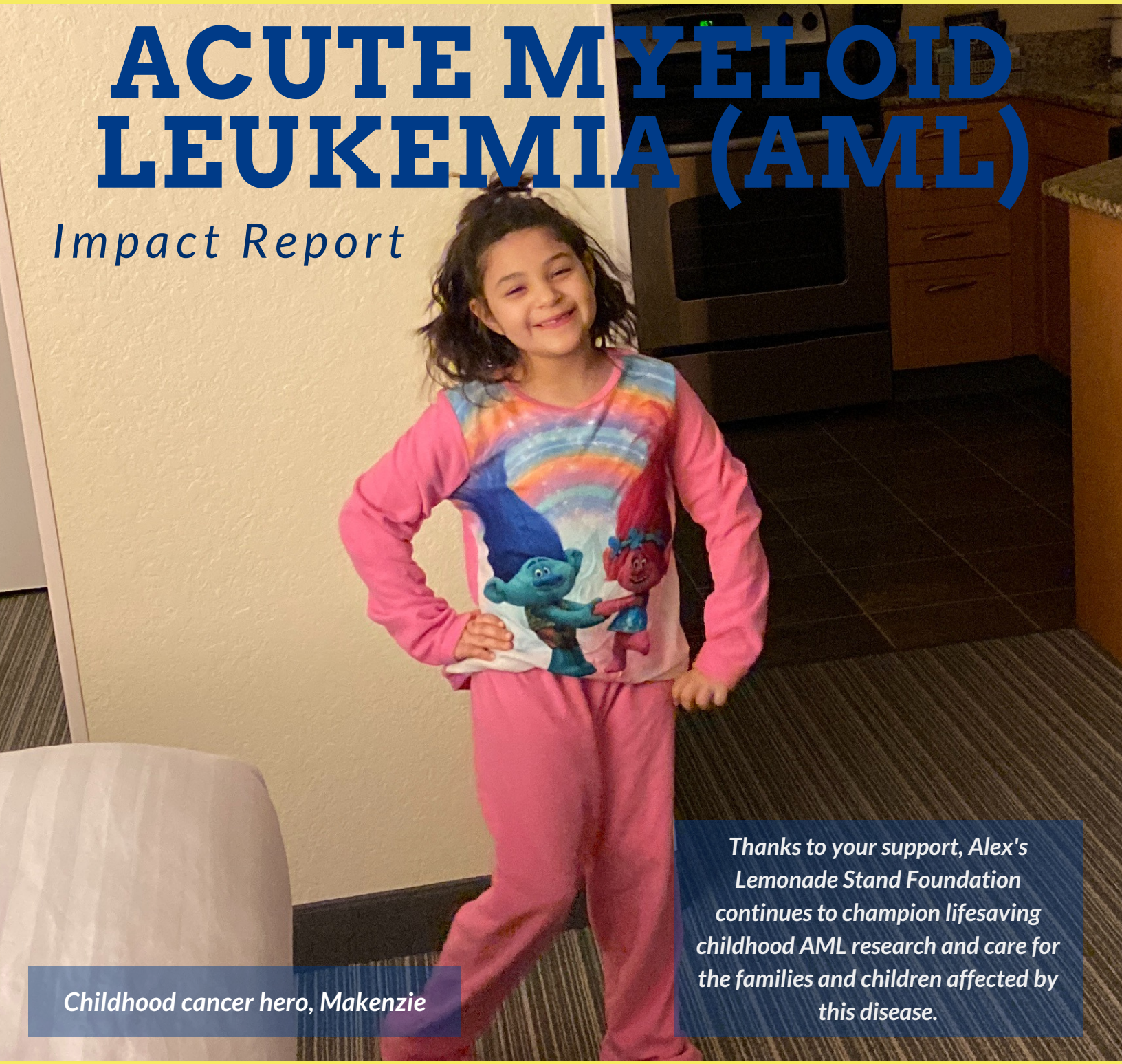
ALEX'S LEMONADE STAND FOUNDATION

ACUTE MYELOID LEUKEMIA (AML)

Impact Report



Childhood cancer hero, Makenzie

Thanks to your support, Alex's Lemonade Stand Foundation continues to champion lifesaving childhood AML research and care for the families and children affected by this disease.

With Gratitude

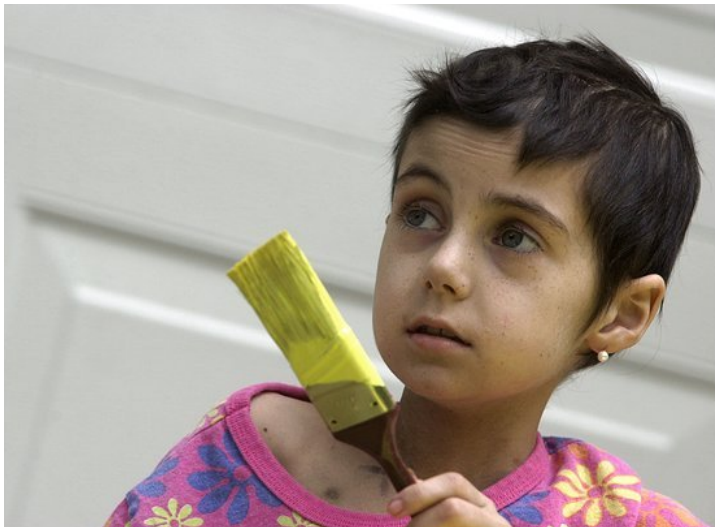
Dear Friend,

The strides that childhood cancer research has made in the past few years are remarkable. New breakthrough treatments have been discovered and approved by the FDA. There are more clinical trials than ever before. Survival rates for certain types of childhood cancers have improved. ALSF remains dedicated to improving treatments for kids with leukemia. We appreciate your support, which is making research like this possible. Thanks to supporters like you believing in research, we are painting a world free of childhood cancer.

Our daughter, Alex, believed that if we all worked together, we could cure childhood cancer. That idea of collaboration is what inspired others to help her reach her $1 million fundraising goal. It's what planted the seed of Alex's Lemonade Stand Foundation. We are always amazed at what can be accomplished when you bring people together. Alex's, scientists, and you – we're all coming together for one common goal: to cure childhood cancer. Thank you for all you continue to do.

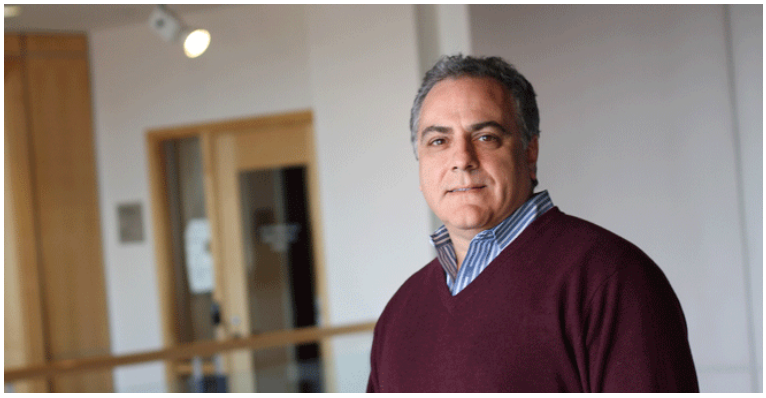
Until there are cures for all kids,

Liz & Jay Scott
Alex's Parents
Co-Executive Directors of
Alex's Lemonade Stand Foundation



Pushing Forward Pediatric Cancer Research in Acute Myeloid Leukemia

Our mission has always been to champion lifesaving childhood cancer research and find cures for all children with cancer.

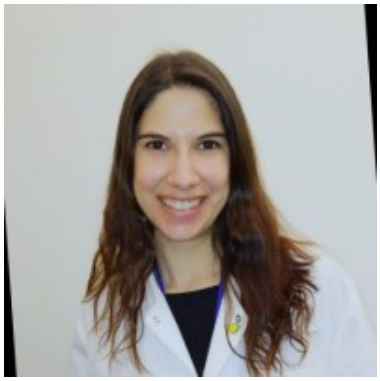


Research Spotlight

Genetic studies have identified a number of genes that when mutated predispose individuals to blood cancers. In addition, these mutations can promote other health complications throughout their lifetime. For example, children that inherit a mutation in the gene RUNX1 have Familial Platelet Disorder, they live with bleeding problems and, in many cases, autoimmune complications. In addition, affected children have a high incidence of blood cancer during their childhood or later in life. Currently, there are not effective therapies to prevent the development of blood cancer. Patients that develop blood cancers with a RUNX1 mutation are treated with chemotherapy and allogeneic stem cell transplantation.

Dr. Lucio Castilla of University of Massachusetts is using his recently awarded Innovation Grant to determine if the correction of the RUNX1 mutation in blood stem cells can restore their normal function and prevent cancer, using a mouse model for Familial Platelet Disorder. These studies may guide future efforts to develop autologous stem cell transplantation protocols that efficiently correct the RUNX1 mutation in the blood stem cells for transplant back into the patient as a curative therapy. The outcome of this study may also serve for the application of similar approaches to other inherited blood cancers.

Targeting RAS-mutated Leukemia



Dr. Sara Canovas Nunes' laboratory, directed by Dr. David Williams, has previously identified Rac (a protein that controls many cellular functions including cell growth and survival) as a promising therapeutic target in refractory leukemias. In recent years, the Williams Lab has partnered with Evotec AG, a drug discovery company based in Germany, to use novel platforms and techniques to identify and develop new inhibitors of Rac. The endpoint of this partnership is to obtain a drug that can be used in a clinical setting for high-risk leukemias and that can improve the prognosis of these aggressive diseases. They've identified a small chemical compound that acts on Rac protein activity and has a good antileukemic action on resistant leukemia cells, in a laboratory setting. Further characterization of this compound could pave the way for new targeted therapy approaches and sustain the preclinical and clinical development of selected compounds already identified by their lab for the treatment of aggressive leukemias.

ALSF Funded Projects in AML

Thanks to you, we have been able to fund outstanding research, leading toward breakthroughs and cures. Read through some of our recently funded projects in Acute Myeloid Leukemia below.

PROJECT TITLE	INSTITUTION / PRINCIPAL INVESTIGATOR(S)	GRANT TYPE
Hematopoietic stem cell function in familial platelet disorder therapy	University of Massachusetts Medical School / Lucio Castilla, PhD	Innovation Grants
Phosphopeptides as shared targets for donor derived T cell therapy of AML	Memorial Sloan-Kettering Cancer Center / Richard O'Reilly, MD	Innovation Grants
Modeling the Initiation and Progression of Down Syndrome Associated Leukemia Using CRISPR/Cas9 at Single Cell Resolution	University Health Network / Elvin Wagenblast, PhD	Young Investigator Grants
Targeting the Rac GTPase Pathway to Sabotage RAS Signaling in RAS-mutated Leukemia	Boston Children's Hospital / Sara Canovas Nunes, PhD	Young Investigator Grants
Barcoding Pediatric Leukemia for Therapeutic Purposes	Boston Children's Hospital / Leonard Zon, MD	Crazy 8 Awards
Assessing Clonal Fitness and Mechanisms of Clonal Evolution in FPD-MN	Memorial Sloan-Kettering Cancer Center / Wenbin Xiao, MD/PhD	RUNX1 Early Career Investigator Grants
Modeling familial platelet disorder associated RUNX1 mutations in mice	University of Massachusetts Medical School / Lucio Castilla, PhD	Familial RUNX1 Research Grants
Single-Cell Profiling of Acute Myeloid Leukemia for High-Resolution Chemo-immunotherapy Target Discovery	Stanford University School of Medicine / Charles Gawad, MD/PhD	Single-cell Pediatric Cancer Atlas Grant
Saturating CRISPR gene body scans in MLL-rearranged leukemia	Beckman Research Institute of City of Hope / Chun-Wei Chen, PhD	Innovation Grants
The Role of Germline Variants in Infant Leukemogenesis	Washington University / Margaret Ferris, MD/PhD	Young Investigator Grants

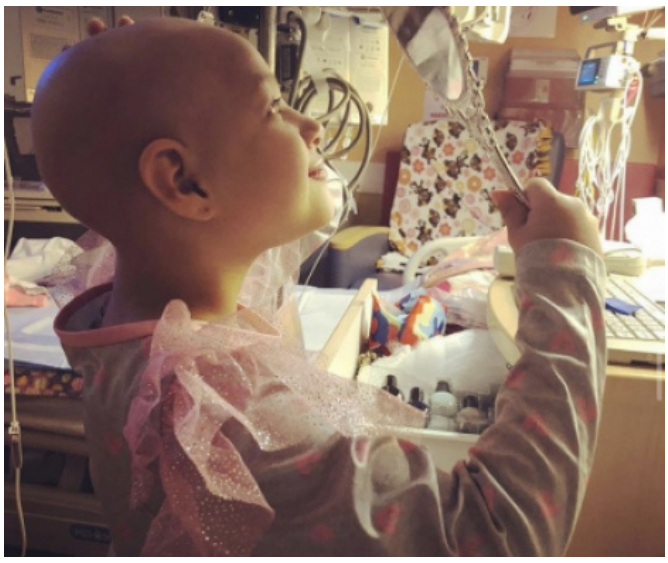
In 2022, we want to fund more high impact, game changing projects like the ones listed above that will target the most deadly childhood cancers and fight for kids affected by childhood cancer around the world. You are the catalyst that makes these cutting-edge research projects possible.

Click here to see a complete list of ALSF funded projects in Acute Myeloid Leukemia

Meet an AML Hero

Part of our mission is to support families in the ways they need it most and empower everyone to help cure childhood cancer.

Meet Makenzie



Makenzie is a bright 8-year-old girl who loves dancing, music and dressing up in high heels and fancy clothes. When she was a year old, she needed a bone marrow transplant to treat her severe combined immunodeficiency. A year later, her family found out that the donor she had been matched with was diagnosed with acute myeloid leukemia (AML).

For three years, her family watched closely for signs of leukemia. Just after the fourth anniversary of her transplant, Makenzie was diagnosed with myelodysplastic syndrome and needed a second bone marrow transplant. Before she could begin the transplant, her cancer had progressed to AML. She began chemotherapy instead.

Makenzie was finally close enough to remission to have her transplant, but it failed. Fortunately, her mother was a match, and Makenzie had her third transplant. Since then, she's been in active treatment to rid her body of the cells from her first transplant.

Every month, Makenzie and her family travel 250 miles from their home for a cycle of chemotherapy. They receive support from Alex's Lemonade Stand Foundation's Travel For Care program in the form of gas cards and hotel stays, which make the travel possible.
"Alex's Lemonade Stand has been our guardian angel. Without them, we would be unable to travel for treatment," said Sheila, Makenzie's mom. "They take helping pediatric cancer families to a whole new level."

When she gets older, Makenzie hopes to have two careers – to become a doctor or nurse and to work at Taco Bell and McDonalds for the free food. Her mom hopes that she will grow up and be healthy.

Thank you for donating to AML research. You are helping fund impactful projects aimed at finding better treatments and cures for kids like Makenzie!