

# ALEX'S LEMONADE STAND FOUNDATION

## ACUTE LYMPHOBLASTIC LEUKEMIA (ALL)

*Impact Report*



*Childhood cancer hero, Marcel*

*Thanks to your support, Alex's  
Lemonade Stand Foundation  
continues to champion lifesaving  
childhood ALL 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 Lymphoblastic Leukemia

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

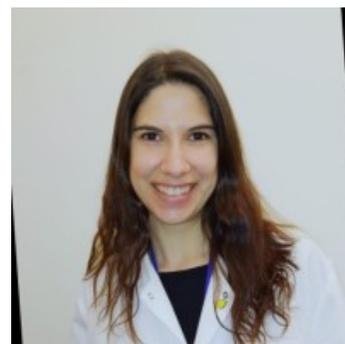


## Research Spotlight

Dr. Yang Ding of the Children's Hospital of Philadelphia is using her recent Young Investigator Award to discover new therapies to improve cure rates for children with Ph-like ALL by analyzing genetic data from patients' leukemia samples. Her ultimate goal is to identify genes that may be very susceptible to new targeted medicines and then test combinations of new medicines in Ph-like ALL cell lines and specialized mouse models. She has examined genetic differences in a large number of leukemia samples from children with ALL and has found previously unidentified drug targets. She predicts that using drugs against these targets will be more effective against leukemia cells while sparing healthy cells. In this project, she will perform laboratory confirmation studies in leukemia cell lines and in patient leukemia cells grown in animal models. The results will help researchers develop effective targeted drug combinations to treat and hopefully cure more children with this high-risk leukemia subtype.

## Targeting RAS-mutated Leukemia

Dr. Sara Canovas Nunes' laboratory, directed by Dr. David Williams, has previously identified Rac 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.



## Using donor-derived genome-edited CAR T cells

Dr. Alice Bertaina of Stanford University and her team have successfully developed a robust system to remove the “attack” gene in the CAR-T cells and replace it with the leukemia-targeting gene. Ongoing studies include methods to ensure the safety of the CAR-T cells. These include: incorporating a safety switch whereby they can program the discard of the engineered CAR-T cells allowing them to fully control their administration, or, adding another gene that can be targeted for discard using drug treatment. Upon completion of funding, Dr. Bertaina expects to be able to manufacture clinical grade adapted CAR-T cells and apply for FDA approval to initiate a clinical trial.

# ALSF Funded Projects in ALL

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 Lymphoblastic Leukemia below.

| PROJECT TITLE   | INSTITUTION / PRINCIPAL INVESTIGATOR(S)  | GRANT TYPE                               |
|---|--|--|
| Novel Synergistic Targets for Combination Therapy in Philadelphia Chromosome-like Acute Lymphoblastic Leukemia            | Children's Hospital of Philadelphia / Yang Ding, MD                                    | 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                           |
| Optimizing TCR $\alpha\beta$ + / CD19+-depleted haploidentical HSCT for ALL using donor-derived genome-edited CAR T cells | Stanford University School of Medicine / Alice Bertaina, MD/PhD                        | Reach Grants                             |
| Generation of CMV-specific CD19 CAR T cells Using Cytokine Capture Followed by Lentiviral Transduction and Expansion      | Research Institute at Nationwide Children's Hospital / Margaret Lamb, MD               | Reach Grants                             |
| Single-cell Profiling of Early T-cell Precursor Acute Lymphoblastic Leukemia  | Children's Hospital of Philadelphia / David Teachey, MD and Kai Tan, PhD               | Single-cell Pediatric Cancer Atlas Grant |
| Single-cell Profiling of Childhood Acute Lymphoblastic Leukemia   | St. Jude Children's Research Hospital / Charles Mullighan, MD and Jeffrey Klco, MD/PhD | Single-cell Pediatric Cancer Atlas Grant |
| Identify Novel Druggable Targets to Treat Philadelphia chromosome-like B-Acute Lymphoblastic Leukemia                     | Children's Hospital of Philadelphia / Wei Tong, PhD                                    | Innovation Grants                        |
| Targeting microenvironment-induced TGFB signaling to overcome drug resistance in acute lymphoblastic leukemia             | St. Jude Children's Research Hospital / Charles Mullighan, MD                          | Innovation Grants                        |
| Novel Therapy of Heterogeneous B-cell Acute Lymphocytic Leukemia by Targeting Convergent Oncogenic Mediators STATs        | University of Alberta / Hasan Uludag, PhD  | Innovation 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 Lymphoblastic Leukemia](#)**

# Meet an ALL 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 Marcel



Marcel is a sweet and caring 6-year-old with energy to spare. He loves basketball and football, especially cheering for the 49ers. His family on the other hand, is focused on cheering for Marcel as he battles acute lymphoblastic leukemia (ALL).

When Marcel was 2 years old, his parents started noticing strange patterns. Marcel would suddenly fall over when walking or running. At first, they thought his shoes were too big, but that quickly changed when Marcel became inconsolable one night. He became feverish and began throwing up. A trip to urgent care and blood work produced no results. It wasn't until he

saw his aunt, an oncology nurse, that he returned to the emergency room with severely low hemoglobin levels. Marcel was diagnosed with ALL.

He soon began chemotherapy and underwent a lumbar puncture every three months. Though he quickly lost his hair, he never lost his positive energy. Through it all, Marcel kept smiling.

His family is grateful that Alex's Lemonade Stand Foundation was able to assist them with gas cards through the Travel For Care program to ensure they never missed an appointment. With so much on their minds, it made a difference to have one less thing to worry about, so they could focus on Marcel.

Today, Marcel is in remission. He's back to being a regular kid, attending school and playing with his Hot Wheels. His parents know there is light at the end of the tunnel. Their wish is to see Marcel grow old and live out his dream of becoming a firefighter.

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