

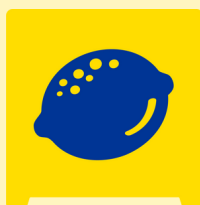


Alex's Lemonade Stand Foundation Neuroblastoma Impact Report





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 Alex's Lemonade Stand Foundation (ALSF) would like to sincerely thank you for your support of Alex's mission to find new treatments and cures for childhood cancers like neuroblastoma.

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 will have to suffer from neuroblastoma.

We are truly honored to fight childhood cancer by your side. Thank you for being the driving force behind life-saving 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

138

Neuroblastoma 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

Meet a **Neuroblastoma Hero**

Name: Philip

Age: 9

Loves: Playing baseball



Philip was 3 years old when doctors told his parents that his neuroblastoma was incurable. Conventional cancer treatments didn't work for Philip, but they never gave up hope for a cure. That hope led them to Children's Hospital of Philadelphia and to Dr. Yael Mossé, an oncology clinician and an ALSF-funded researcher.

Dr. Mossé had an open clinical trial for a targeted drug to help kids like Philip, which uses a drug called lorlatinib. Lorlatinib was previously approved by the FDA for the treatment of small cell lung cancer in adults, but before Dr. Mossé's trial, it had not been tested for use in the treatment of neuroblastoma. Philip enrolled in the trial in 2019.

For Philip, treatment on lorlatinib came with several benefits. He could take the drug in a pill format versus intravenously like his previous treatments, and the side effects were relatively manageable and mild, as compared to other traditional neuroblastoma treatments like chemotherapy and radiation. Philip was able to be a regular kid while on treatment.

By summer 2022, Philip hit his first home run in Little League, entirely cancer-free. His parents are forever grateful to Dr. Mossé and the trial that gave their son hope for a future.

"Philip wouldn't be here," said his mom, Wendy. "This was his last shot at helping him survive."

Research Spotlight: New Projects in Neuroblastoma

Arginine Depletion with DFMO in High-Risk Neuroblastoma

Christina Turn, MD, Children's Hospital of Philadelphia

Dr. Christina Turn and her team recently made the striking discovery that DFMO and the proline/arginine deficient diet can cure mice with neuroblastoma. As a result of this discovery, Dr. Turn aims to specifically clarify the role of arginine in blocking neuroblastoma growth. One of her goals is to determine whether a combination of DFMO and a arginine-deficient diet has similar survival results. Her other goal is to characterize the effects of arginine depletion and DFMO upon the immune microenvironment and to determine how it affects the tumor's populations of T cells and NK cells. Dr. Turn and her team will evaluate the effects of this treatment on the immune microenvironment, signaling proteins, and immune cells. If effective, this treatment combination could be translated into a clinical therapeutic regimen to improve the survival of children with high-risk neuroblastoma.



Targeting Oncogenic N-MYC Complexes in High-Risk Neuroblastoma

Bo Qiu, MD/PhD, University of California San Francisco

Dr. Bo Qiu's goal first and foremost is to identify how the MYCN gene drives aggressive neuroblastoma tumor growth and find new approaches to treat this group of patients. When genes are mutated in cancer cells, they lead to changes in the activity of proteins, which are the machines that carry out all the functions of the cell. Increased levels of MYCN in cancer cells recruit other protein machinery to drive uncontrolled cancer growth. Dr. Qiu has developed a sensitive method to identify all the proteins that are recruited by MYCN in cancer cells. His initial efforts have identified two key proteins that MYCN recruits to drive aggressive cancer growth (named FUS and PRMT5). Both proteins have drugs that are being tested in clinical trials for human diseases, including neurodegenerative diseases and adult cancers. However, there are no trials testing these drugs in pediatric cancer. Dr. Qiu's planned experiments will rigorously test how these proteins drive cancer and build a case to test these drugs in children afflicted by high-risk neuroblastoma.





Where Are They Now?

Neuroblastoma Breakthrough: ALSF-funded researcher finds potential treatment for high-risk neuroblastoma

Dr. Yael Mossé of the Children's Hospital of Philadelphia was first awarded a grant from ALSF in 2013 to study preclinical development of a targeted therapy for neuroblastoma. Fast forward 10 years later, Dr. Mossé is now in her third year of a Crazy 8 Award focused on drugging MYCN, a protein which is the cause of aggressive cancers like neuroblastoma.

Dr. Mossé has dedicated her career to researching and treating neuroblastoma. She has been funded several times by ALSF, leading to several critical findings for kids facing neuroblastoma. Dr. Mossé had an open clinical trial for a targeted drug. The clinical trial is funded in part by ALSF and conducted by a group of researchers at Children's Hospital of Philadelphia (CHOP), Winship Cancer Institute of Emory University, and the New Approaches to Neuroblastoma Therapy (NANT) consortium to test lorlatinib as a single treatment agent in children and adults in combination with chemotherapy in children. The patients enrolled on the trial all had relapsed or refractory neuroblastoma and had received previously unsuccessful treatment for their disease.

Now, the drug will be available to newly diagnosed children as a frontline treatment, bringing it one step closer to FDA approval.



Thank You

for all you do to help kids with cancer!

