ALEX'S LEMONADE STAND FOUNDATION

MEDULLOBLASTOMA

Impact Report

Childhood cancer hero, Abby

Thanks to your support, Alex's Lemonade Stand Foundation continues to champion lifesaving childhood medulloblastoma 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 medulloblastoma. 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 Medulloblastoma Research

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

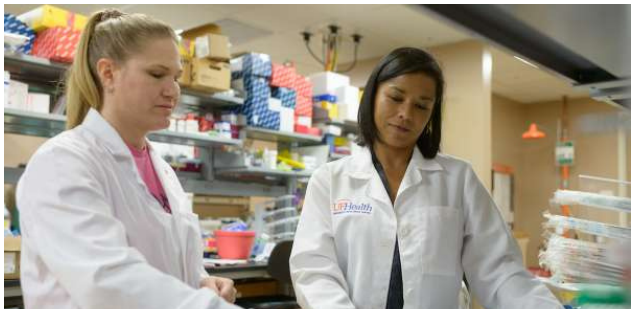
Research Spotlight

Dr. Charles Mullighan of St. Jude's Children's Hospital was awarded a highly esteemed Crazy 8 Award in early 2020 with the goal of expanding and utilizing a library of molecular glues to treat some of the deadliest childhood cancers like medulloblastoma and leukemia. Glue sounds like an unlikely tool for a pediatric oncology researcher. But for Dr. Charles Mullighan and his Crazy 8 project team, molecular glue offers the promise for effective treatments and cures for children with brain tumors and leukemia.

"Small molecule protein degraders, often referred to as molecular glues, offer the tantalizing prospect of targeting currently undruggable oncoproteins." said Dr. Rankovic, the chemistry lead of this multidisciplinary team.

Molecular glues are a recent concept in drug discovery, and their application to childhood cancer hasn't yet been fully explored. The molecular glue is designed to bind to a specific transcription factor that drives tumor growth, and then directs the cancer cell to break it down. Since transcription factors are master regulators of cell growth and commonly mutated in leukemia and medulloblastoma, by degrading mutant transcription factors, the cancer cell dies. The result is a targeted therapeutic that can attack cancer with potentially fewer side effects to the patient. For medulloblastoma and leukemias, which combined make up the deadliest types of childhood cancers, molecular glues hold the promise for more effective and safer treatments and cures.

Reversing treatment resistance to PD-1 blockade in brain tumors



Dr. Catherine Flores of the University of Florida recently wrapped up her Innovation Grant in which she developed a clinically applicable method of overcoming treatment resistance to αPD-1 in multiple refractory brain tumors. Dr. Flores and her team employed two pediatric brain cancer models, high grade glioma and medulloblastoma which are refractory to PD-1 monotherapy and standard treatments alone. Their previous studies determined that HSC + PD-1 combinatorial therapy lead to efficacy in these models. They determined that HSC + PD-1 actually leads to the decreased overall amounts of suppressive cells in the tumor by preventing their migration into the tumor site, thus decreasing the overall immunosuppression in the tumor. The immunosuppressive tumor microenvironment in brain cancers is one of the major hurdles in therapies against these diseases. Their findings are paramount in finding hope for children with brain malignancies.

ALSF Funded Projects in Medulloblastoma

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 medulloblastoma below.

PROJECT TITLE	INSTITUTION / PRINCIPAL INVESTIGATOR(S)	GRANT TYPE
Small Molecule Degraders for Targeting Transcription Factor Drivers of Childhood Cancers	St. Jude Children's Research Hospital / Charles G. Mullighan, MBBS(Hons), MSc, MD, FRACP, FRCPA	Crazy 8 Awards
Drugging MYCN	Children's Hospital of Philadelphia / Yael Mossé, MD	Crazy 8 Awards
Single-cell Gene Expression and Cytosine Modification Profiling in Pediatric Central Nervous System Tumors	Dartmouth College / Brock Christensen, PhD	Single-cell Pediatric Cancer Atlas Grant
The use of hematopoietic stem and progenitor cells to reverse treatment resistance to PD-1 blockade	University of Florida / Catherine Flores, PhD	Innovation Grants
The anti-tumor immune microenvironment in the Sonic Hedgehog subclass of medulloblastoma	Emory University / Anna Kenney, PhD	Innovation Grants
Targeting symmetric division in pediatric cancers	Dana-Farber Cancer Institute / Rosalind Segal, MD/PhD	Innovation Grants
Targeting TGFb Pathway Dependencies in Group 3 Medulloblastoma	University of California San Francisco / Zulekha Qadeer, PhD	Young Investigator Grants
Characterization of Chromatin Landscapes to Identify Therapeutic Vulnerabilities in Diffuse Midline Gliomas.	Fred Hutchinson Cancer Research Center / Jay Sarthy, MD/PhD	Young Investigator Grants
Targeting the Surface-ome of Embryonal Brain Tumors	Institut Curie / Olivier Ayrault, PhD	Crazy 8 Pilots
Identifying a Therapeutic Partner for TAK228 for Pediatric Phase II Brain Tumor Studies	The Johns Hopkins University School of Medicine / Eric H. Raabe, MD/PhD	Reach 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. Thanks to you, we are one step closer to a world where cures are a reality.

Click here to see a complete list of ALSF funded projects in Medulloblastoma

Meet a Medulloblastoma 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 Abby

Abby is an incredibly strong and brave 9-year-old who loves unicorns, hunting for bugs and playing with Legos. Despite battling cancer, she often tells her mom how much she loves her life.

When Abby had trouble focusing in school, her parents suspected she had ADHD. A few months passed and Abby began experiencing headaches so severe she would throw up. She was referred to a neurologist who wasn't concerned by her symptoms. Luckily, Abby's pediatrician sent her to the emergency room for a CT scan just to be safe. Abby was diagnosed with medulloblastoma.

Surgery removed her brain tumor, and unfortunately, cost her control over right hand along with impaired balance and coordination. Her treatment has been a long road, filled with 31 rounds of brain and spinal radiation, 24 rounds of chemotherapy, losing her hair 5 times, and nearly a full year without school. Still, Abby's bravery and strength never fail her – she's even learned to write with her left hand.

Alex's Lemonade Stand Foundation (ALSF) have given Abby's family hope for safer treatments and a cure. They take comfort in the fact that ALSF is changing the childhood cancer research game. Her family has two wishes for the future: that no parent will have to suffer the loss of their child to cancer and that Abby will get to fulfill her dream of owning an animal sanctuary.

Thank you for donating to medulloblastoma research. You're helping fund impactful projects aimed at finding better treatments and cures for kids like Abby!