

Alex's Lemonade Stand Foundation Spinal Cord Tumors Impact Report



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,

Thanks to your thoughtful support, we have been able to find new discoveries for childhood cancer treatment. ALSF-funded researchers continue to develop breakthrough treatments, lead new clinical trials, and publish their findings in peer-reviewed journals. Thanks to you, we were able to make this progress, which has positively impacted the lives of children with spinal cord tumors.

Our daughter, Alexandra "Alex" Scott, 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. Her idea is the reason we are able to do what we do now. We are always amazed at what can be accomplished when you bring people together. The Foundation, scientists and you — we're all coming together for one common goal: to cure childhood cancer. Thank you for all you do in the fight against childhood cancer.

Until there are cures for all kids,

Liz & Jay Scott

Alex's Parents & Co-Executive Directors

Alex's Lemonade Stand Foundation



Thanks to Supporters Like You



Childhood Cancer Research Projects Have Been Funded Since Our Founding



The ALSF name is so well-respected in the research community, it provides a marker of success that is extremely helpful when I go to apply for other grants."

— Dr. Adam Wolpaw, Children's Hospital of Philadelphia

The 'high-risk, high-reward' studies that
ALSF supported are some of the very studies
that best represent me, our science, and serve
as the platform for the work we have ongoing
and propose for the future. It doesn't escape
me that the support from ALSF has been
instrumental, and I extend my deep
appreciation to ALSF for supporting our
science from the very beginning."

— Dr. Cigall Kadoch,

Dana-Farber Cancer Institute

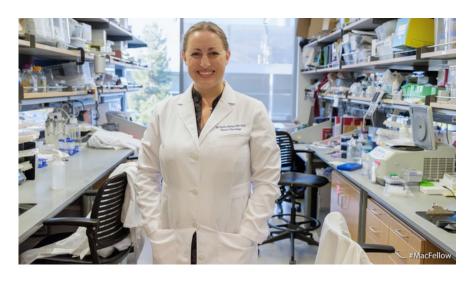




Research

Spotlight

As a young trainee, many people gave Dr. Michelle Monje, ALSF grantee from Stanford University the (unsolicited) advice that one cannot have a big career in medicine or science and also have children.



Fortunately, she ignored the advice. Dr. Monje (mother of four) was awarded a Catalyst Grant from ALSF early this year. This grant is a \$1 million commitment over two years to study CAR T cell immunotherapy for spinal cord diffuse midline gliomas in a Phase 1 clinical trial. Diffuse midline gliomas are inoperable, lethal, high-grade central nervous system tumors primarily affecting children and young adults.

Dr. Monje discovered that DMGs, which are characterized by the presence of a specific mutation called H3K27M+, exhibit high levels of a signal called GD2 and that immune cells engineered to recognize this signal called chimeric antigen receptor T cells (GD2-CAR T cells) mediate impressive anti-tumor effects in numerous patient-derived mouse models of DMG, including spinal cord DMG. Based on her compelling laboratory findings, her current research delivers a bench-to-bedside translation of GD2-CART therapy for children and young adults with H3K27M+ DMGs through conduct of a Phase I clinical trial. Dr. Monje is also undertaking a bedside-to-bench translation focused on learning as much as possible from the clinical course and outcomes of patients enrolled in this Phase I trial in order to optimize this promising immunotherapeutic approach. The Phase I trial includes 4 arms for individuals with pontine and spinal cord DMGs and for CAR T cell administration intravenously (IV) or directly into the central nervous system (intracerebroventricularly, ICV). Her cutting-edge proposal focuses on the spinal cord DMG arms of the clinical trial and on optimizing immunotherapy for spinal cord tumors in general, as her early results suggest important differences in the tumor response to CAR T cell therapy in the brain and spinal cord.

"ALSF helped launch my research program 10 years ago and has supported my lab at every step along the way. Their investment in childhood cancer research has transformed the field and nurtured the development of a generation of childhood cancer researchers," said Dr. Monje.



ALSF-Funded Projects in Spinal Cord Tumors

Thanks to you, we have been able to fund outstanding research, leading towards breakthroughs and cures. Read through some of our recently funded research projects in spinal cord tumors below.

PROJECT TITLE	INSTITUTION / PRINCIPAL INVESTIGATOR(S)	GRANT TYPE
GD2-directed CAR T cell therapy for H3K27M+ spinal cord diffuse midline glioma	Board of Trustees of the Leland Stanford Junior University / Michelle Monje	Catalyst Grants
Targeting Spinal Ependymoma and Other Gliomas Using Novel Genetically Defined Models	The Johns Hopkins University School of Medicine / Charles Eberhart, MD, PhD	Catalyst Grants
3-D Printed Self-fitting Shape Memory Bone Grafts for Smart Pediatric Skeletal Reconstruction	University of Massachusetts Medical School / Jie Song, PhD	Innovation Grants
Role of Protocadherin-9 in Enabling Leukemia Cell Colonization of the CNS	National Jewish Health / Jordan Jacobelli, PhD	Innovation Grants
Developing and Distributing a Pediatric Spinal Cord High-grade Glioma Model	Stanford University / Michelle Monje, MD/PhD	Innovation Grants
Preclinical Testing of Candidate Therapeutics in a Pediatric Spinal Cord High-grade Glioma Model	Stanford University / Michelle Monje, MD/PhD	Innovation Grants
Modeling Pediatric Spinal Gliomas Using Murine Spinal Neural Stem Cells	The Johns Hopkins University School of Medicine / Charles Eberhart, MD/PhD & Eric Raabe MD/PhD	Innovation Grants
Developing a Novel Tumor Model to Screen for New Therapies for Spinal Ependymoma	The Johns Hopkins University School of Medicine / Linda Resar, MD & Richard Schlegel, MD, PhD	Innovation Grants

Click here to see a complete list of ALSF-funded projects in spinal cord tumors



Meet Childhood Cancer Heroes

One day, Laney's left arm started itching so badly that she scratched until it bled. Six months later, her arm went limp. At first, her doctor suspected it was caused by a stroke. But then, an MRI scan showed the tip of a tumor on her spinal cord. She and her family were rushed to Children's Hospital of Philadelphia where Laney went into emergency removal surgery.

Since then, Laney has endured three major and two minor surgeries, plus two failed chemotherapy regiments with 63 counts of infusion. Once her family learned

that the tumor was intertwined with her spinal cord and chemotherapy no longer an option, Laney had to go through 25 rounds of proton radiation. Because of the intensity and variety in treatment, Laney now has major left side deficits, and uses braces, including a wheelchair on some days.

To Laney's mom Nicole, ALSF means happiness and hope – happiness in seeing how many people are involved, and hope that a cure for childhood cancer will be found.



When Wyatt was 15 months old, he was diagnosed with a rare ganglioglioma inside his spinal cord. Three months earlier Wyatt frequently cried, vomited, lost his balance, and choked while eating. Each day, the symptoms were gradually getting worse. His family took him to the emergency room several times before an MRI was finally done. His brain and spinal cord were so swollen that he had to wait to get surgery.

After the surgery, and many hours of therapy to relearn how to swallow, walk and talk, Wyatt was doing well. But gradually, his symptoms emerged again.

Another surgery was needed to remove the regrown tumor followed by therapy once again. Today, Wyatt does not have much feeling in his arms and hands, but he continues to amaze everyone who meets him by doing so many things other kids his age can do. He continues to undergo an MRI every six months to monitor possible growth of what might be left of his tumor.











Thank You

for all you do to help kids with cancer!

