

ALEX'S LEMONADE STAND FOUNDATION

IMPACT REPORT

Diffuse Intrinsic Pontine Gliomas

Mariella Filbin, MD/PhD, was selected as an ALSF Crazy 8 pilot grantee. She is making groundbreaking progress in targeting DIPG, a cancer that has a 0% survival rate and is a high priority for ALSF funding.

Thanks to your support, Alex's Lemonade Stand Foundation (ALSF) has continued to champion lifesaving childhood cancer research in Diffuse Intrinsic Pontine Gliomas (DIPG) and care for the families and children affected by this disease.

Pushing Research Forward In Diffuse Intrinsic Pontine Gliomas (DIPG)

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



Research Spotlight

Dr. Costas Lyssiotis of the University of Michigan received an ALSF Innovation Grant award to therapeutically target the disrupted metabolic state in diffuse intrinsic pontine gliomas (DIPG). Dr. Lyssiotis plans to expand and translate these studies by determining the molecular underpinnings that lead to the profound sensitivity of DIPG cells to ferroptosis, and by evaluating the anti-tumor

activity of ferroptosis in human patient-derived DIPG tumor models. Unlike cancer cells, normal cells readily tolerate inhibition of many of the redox control nodes that promote ferroptosis (an iron-dependent form of cell death mediated by the accumulation of toxic lipid peroxides) in DIPG cells. Results from this research will uncover novel druggable targets for DIPG therapy and evaluate ferroptosis as a treatment regimen that can proceed to clinical trials for DIPG patients.

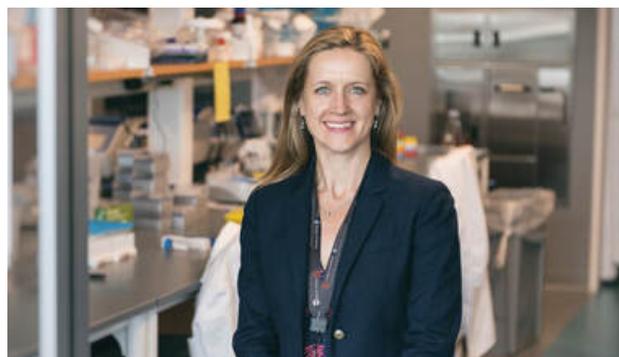
Targeting a Novel Epigenetic Signature in DIPG

Dr. Danny Reinberg is using his Reach Grant to strategically target novel epigenetic signatures in DIPG. Dr. Reinberg and his team have developed preliminary compounds that block the ability of proteins that specifically bind epigenetic modification H3K36me2. They are working to develop compounds that inhibit the enzymes that cause the elevation of this modification. Further in vivo testing of these compounds in transgenic mice engineered with DIPG tumors will be carried out at NYU. These compounds are very promising therapeutics for the treatment of DIPG.



Dissecting Microenvironmental Interactions in DIPGs That Promote Tumor Growth

Dr. Mariella Filbin of Dana-Farber Cancer Institute used her Crazy 8 pilot funding to conduct single-cell and single-nucleus RNA-seq analyses to discover developmental pathways hijacked by tumor cells in different classes of high-grade glioma and



characterize lines of communication between tumor cells and their surrounding microenvironmental cells. She enrolled nine of the total 10 patients proposed for this Pilot Grant and successfully sorted all of their tumors into 96-well plates.

[More about ALSF-funded projects in DIPG can be found here: AlexsLemonade.org/Childhood-Cancer/Type/DIPG](https://AlexsLemonade.org/Childhood-Cancer/Type/DIPG)

Meet A DIPG 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 Kayne



Kayne was kind, compassionate and funny. He loved animals, especially dogs, and dreamed of becoming a Doctor of Veterinary Medicine. Kayne was active in competitive swimming since he was 5 years old and enjoyed boogie boarding and surfing.

In October 2016, Kayne noticed that his left eye was not fully closing when he was in the ocean. He also experienced other symptoms, like fatigue, mild hearing loss and an inability to move the left side of his face. His doctor ordered a full brain MRI and discovered a mass lesion near

Kayne's brain stem. Kayne was diagnosed with diffuse intrinsic pontine glioma (DIPG).

Once in the hospital, he met with the pediatric neuro-oncology and radiology oncology teams to develop a treatment plan, which included six weeks of targeted radiation followed by a clinical trial. Kayne responded well to radiation and many of his symptoms were reduced. He also enrolled in the Ribociclib clinical trial led by ALSF's Quality of Life and Care Grantee, Mariko DeWire-Schottmiller, at Cincinnati Children's Hospital Medical Center.

In fall of 2017, Kayne attended Louisiana State University, majoring in the College of Agriculture and Animal Sciences, following his dream of becoming a veterinarian. Sadly, he passed away in November 2017.

Before passing, Kayne signed a directive to donate his tumor to scientific research. "I hope that with all the people that know my name and my diagnosis, I can be a beacon of inspiration worldwide. Please educate yourself and others about DIPG and help us fight for and develop a cure. Every single person makes a difference," said Kayne.

Kayne's family hopes that by joining with ALSF they can spread awareness about pediatric cancer, and more importantly, raise funds for DIPG research to find cures for future children.

Thank you for supporting DIPG research! You're giving hope to childhood cancer heroes!