

ALEX'S LEMONADE STAND FOUNDATION

IMPACT REPORT

Rhabdomyosarcoma



Childhood cancer hero Abigail, featured above, and her family are committed to supporting childhood cancer research.

Thanks to your support, Alex's Lemonade Stand Foundation has continued to champion lifesaving rhabdomyosarcoma research and care for childhood cancer patients and their families.

Pushing Forward Rhabdomyosarcoma Research

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



Research Spotlight

Dr. Genevieve Kendall of Nationwide Children's Hospital has used her 'A' Award Grant to integrate the human forms of rhabdomyosarcoma cancer genes into the zebrafish genome and study the processes for developing tumors. She has found that genetic suppression of two genes inhibits the rhabdomyosarcoma zebrafish phenotype, indicating that these two genes are candidates for chemical inhibition and represent new therapeutic opportunities. Dr. Kendall was awarded

the ALSF 'A' Award during a critical juncture in her career, propelling her from a postdoctoral position to a tenure-track Assistant Professor position. She has published her research in bioRxiv and was invited to speak at two national lectures on developmental programs for rhabdomyosarcoma tumorigenesis.

Investigation using single-cell sequencing

Dr. Anand Patel of St. Jude's Children's Research Hospital is using his Young Investigator Grant to create an experimental model from patient rhabdomyosarcoma tumors that are expanded in mice in order to understand how these tumors occur. These studies will establish a cell-by-cell atlas of rhabdomyosarcoma and will allow researchers to address which cells within tumors survive therapy. The findings from his study have the potential to identify new therapeutic avenues for this disease.



Targeting MDM2 for rhabdomyosarcoma therapy

Dr. Dawn Chandler of Nationwide Children's Hospital is using her Innovation Grant to understand the way in which tumors become metastatic and resistant to drug treatment, so that she and her team can interfere to kill the tumor. Inactivation of genes that regulate cell growth can lead to uncontrolled growth in cells and eventually can cause tumors. Genes that regulate important tumor suppressor genes can also play a role in cancer protection. One known tumor suppressor gene, p53, is regulated by a number of other genes. Her work has shown that an important negative regulator, MDM2, is altered by a process called alternative splicing by which sections of the gene are differentially excluded to make MDM2-ALT1 and thus promote the formation of RMS tumors. Her work is currently testing the hypotheses that preventing alternative splicing of the MDM2 will prevent cancer growth and progression to provide a therapy for RMS.



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

Meet A Rhabdomyosarcoma 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 Abigail



Abigail loves to play with her four brothers, read and ride her tricycle. She's also a huge fan of the movie *Frozen* and insists on watching it during every treatment and hospitalization. She has an infectious laugh and irresistible smile.

Abigail was experiencing some unexplained bleeding that prompted a trip to the doctor. She was initially diagnosed with an infection, but when her symptoms persisted after taking antibiotics, Abigail needed an ultrasound. The doctor discovered a 4x2 centimeter mass in her bladder.

Abigail was diagnosed with embryonal rhabdomyosarcoma. Her treatment plan included 20 consecutive radiation treatments, 43 weeks of chemotherapy, a series of biopsies, and a surgery to remove a portion of the tissue around the mass.

To her family, Abigail is a hero. Her three older brothers are involved with ALSF's SuperSibs program. They are excited to receive their own packages in the mail on a regular basis. SuperSibs has allowed Abigail's brothers to feel that they have a special place within the family throughout Abigail's treatment.

Her family also hosted a lemonade stand to support pediatric cancer research to improve survival rates for kids all over the world. They couldn't believe the amazing support of their community. They exceeded their initial fundraising goal of \$100, raising more than \$500! "Always try your best to help people in need," said Zach, Abigail's brother.

Abigail's family hopes that she will continue to live a full, active life and share her hope with others.

SuperSibs

The SuperSibs Comfort & Care Mailing Programs send age-appropriate support over two years to children ages 4-18 residing in the U.S. who have experienced the childhood cancer diagnosis of a sibling.

Thank you for supporting rhabdomyosarcoma research. You're giving hope to childhood cancer heroes like Abigail!