

# ALEX'S LEMONADE STAND FOUNDATION

# IMPACT REPORT

*Neuroblastoma*

*Childhood cancer hero Lilly, featured above, was rushed to Children's Hospital of Philadelphia (CHOP), an Alex's Lemonade Stand Foundation Center of Excellence, after receiving a neuroblastoma diagnosis.*

*Thanks to your support, Alex's Lemonade Stand Foundation has continued to champion lifesaving childhood neuroblastoma research and care for the families and children affected by this disease.*

# Pushing Forward Pediatric Cancer Research in Neuroblastoma

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



## Research Spotlight

Dr. Yael Mosse of Children's Hospital of Philadelphia received a Crazy 8 Pilot Grant in order to explore innovative approaches and utilize cutting-edge technologies to target MYCN, an ideal drug target in neuroblastoma. Since the original description in 1983 of MYCN amplification as the first described oncogene in human cancer from studies of neuroblastoma cell lines, an intense focus

has been placed on targeting this cancer gene. MYCN is one of the few proteins that is always upregulated in cancer. The seemingly insurmountable challenge of targeting MYCN is now evolving as researchers' knowledge of MYCN biology and recent technological advances are providing an exceptional opportunity to reach the goal of targeting MYCN tumors. Dr. Mosse's Crazy 8 Pilot Grant utilized cutting-edge technologies in order to attack the proteins encoded by MYCN as well as MYCN itself. Her work has pushed forward efforts to create effective therapies for MYCN-addicted cancers.

## Selectively targeting EP300 for neuroblastoma therapy

Dr. Jun Qi of Dana-Farber Cancer Institute is using his Innovation Grant to selectively target the novel oncoprotein, EP300, using small molecules for neuroblastoma therapy. Dr. Qi and his team are using a powerful tool - a degrader molecule that can stop the growth of neuroblastoma cells causing little to no toxicity to normal tissues. They are using this to understand why EP300 is a dependency in neuroblastoma using cancer cells and animal models with the ultimate goal of establishing a pre-clinical rationale. They are specifically designing a drug to address the dire needs of pediatric cancers, contrary to many industry drugs that are designed for adult cancers.



## Pushing forward immunotherapies of neuroblastoma and osteosarcoma

Dr. Robbie Majzner of Stanford University is using his 'A' Award to study the synergistic activity of anti-GD2 antibodies and CD47-blockades for immunotherapy of neuroblastoma and osteosarcoma. A large percentage of neuroblastoma patients who receive anti-GD2 immunotherapy relapse. Dr. Majzner and his team have found that combining anti-GD2 therapy with an antibody that blocks CD47, a 'Don't Eat Me Signal' expressed by cancer, unleashes immune cells to phagocytose (eat) tumor cells. They are currently translating this combination to clinical trials in children. This project focuses on understanding the biologic basis for this enhanced efficacy, so that they can find future combination therapies to further extend the reach of immunotherapy in pediatric solid tumors.

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

# Meet A Neuroblastoma 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 Lilly



Lilly is a 4-year-old firecracker. She loves learning, especially colors, shapes, ABCs and counting.

As an infant, Lilly was often sick and was misdiagnosed with a virus and asthma. When Lilly was having difficulty breathing, her mom, Nicole, rushed her to the emergency room. An x-ray showed a large mass in her chest. Lilly was diagnosed with neuroblastoma and rushed to Children's Hospital of Philadelphia (CHOP), an Alex's Lemonade Stand Foundation Center of Excellence, to start treatment.

The next day, Lilly had surgery to remove the mass in her chest. Unfortunately, the surgery was unsuccessful because the mass was too close to her lungs and spine.

Lilly was so weak that she was placed in a medically induced coma for two weeks. She lost half her body weight and had to learn to eat and walk again with physical and occupational therapy.

Emergency chemotherapy started and continued throughout that summer. Lilly lost her hair but never her smile. Chemotherapy shrunk her tumor 50% and she had the chance to recover for a few months. Unfortunately, Lilly relapsed and required another surgery. Again, the location of Lilly's tumor meant that it could not all be removed.

To date, Lilly has endured countless pokes, monthly anesthesia, injections, transfusions, chemo and severe anxiety. She is a tiny little girl who overcame and went through more trauma than most adults. She is a superhero!

Recently, Lilly's doctors were concerned with an abnormality in her scans. Her mother, Nicole, hopes that Lilly's future scans will show no evidence of disease. Nicole says, "Years from now, we cannot wait to tell Lilly how brave she was while celebrating survivorship."

***Thank you for supporting neuroblastoma research. You're giving hope to childhood cancer heroes like Lilly!***