

IF A CHILD'S LIFE WAS IN DANGER, YOU WOULD DO SOMETHING, WOULDN'T YOU?



Children's Brain
Tumor Project
powered by families



Weill Cornell
Medicine

Fall 2021

Hip Hop for Hope Honoring Tim & Barbara Michels Raises \$800K Hosted by the Children's Brain Tumor Project Foundation, Dinner Chair Tara Lipton



The "No Laughing Matter" comedy night fundraiser, hosted by the Children's Brain Tumor Project Foundation on September 24 at the New York Athletic Club, was an amazing comeback. Generous ticketholders and donors contributed to an event gross of more than \$800,000 to benefit pediatric brain tumor research at Weill Cornell Medicine.

After a painful pause in 2020, Drs Jeffrey Greenfield and Mark Souweidane were thrilled to announce the return of the annual "No Laughing Matter" fundraiser, the single largest source of income for the Children's Brain Tumor Project lab. The theme for this year's event was "Hip Hop for Hope" featuring an unforgettable performance by Freestyle Love Supreme just days before the improv troupe, founded by Lin Manuel Miranda, returned to Broadway.

"I saw Freestyle Love Supreme perform with my family just before the shutdown, and we were all blown away by how funny and creative the show was," said dinner chair Tara Lipton, mom to Walker who was diagnosed with medulloblastoma in 2016. "When it came time to plan

the comedy night fundraiser this year, I knew we had to ask them to perform. I am so grateful for their willingness to support this important cause that is so close to my heart."

Rather than appointing a traditional event emcee, Drs. Greenfield and Souweidane chose to have their young patients present throughout the evening. It was deeply touching for the audience to hear from the children and young adults who benefit from their donations. A humorous introduction by Tara and Walker Lipton (11), followed by an unexpected rap performed by Dr. Greenfield kicked off the show. After Freestyle Love Supreme captivated the audience with their hilarious improv, a video from patient Charlie Dubois (16) announced the return of Dr. Souweidane, who then presented Tim and Barbara Michels with the second annual "Liz Laugh Love" award. The award acknowledged their generous pledge to endow the Michels Family Professorship in Pediatric Neurological Surgery. [Read more about the endowment on page 6.](#)

Tim was joined on stage by his daughter, Sophie (20)—a patient of Dr. Mark Souweidane. Sophie spoke elegantly about her own experience with a brain tumor, her vision for the evolution of treatment for kids with cancer, and the importance of research funding.

Isabelle Melnick (13) appeared next to share her inspiring story and to introduce the research appeal for the expansion of the precision medicine program at the Children's Brain Tumor Project. NBC news anchor Tom Llamas conducted an energetic live auction before Stuart Macnaughton (14) took the stage to introduce the final speaker, Jessica Schwartz (20). She blew everyone away with her poise and optimism despite the recent obstacles she has faced. The stage program ended with an adorable montage of "thank you" messages recorded from patients William Harrison (18), Taylor Trimarchi (5), and Enzo Peloquin (7).

Tremendous thanks to the sponsors, ticketholders, and donors who made this event a success. The funding is absolutely essential to ensure this great work continues.



From Left: (1) Dr. Souweidane, Dr. Greenfield, and Kailya Mullady of Freestyle Love Supreme, (2) Walker and Tara Lipton (3) Freestyle Love Supreme, (4) Jessica Schwartz

First-Ever Phase 1 Clinical Trial for Choroid Plexus Carcinoma (CPC) is Open for Enrollment

Dr. Mark Souweidane's Phase 1 clinical trial using intra-arterial (IA) chemotherapy prior to second-look surgery is open for enrollment for patients diagnosed with choroid plexus carcinoma (CPC), the first such trial to specifically address this rare diagnosis. IA chemotherapy is a minimally invasive procedure that delivers chemotherapy directly to the tumor via the dominant artery, thus greatly reducing systemic toxicity while enabling higher drug concentrations.

Choroid plexus carcinoma is a rare malignant brain tumor that develops in the ventricles of the brain, most often in children. Successful resection surgery directly correlates with more successful outcomes among the patient population, which is why this clinical trial explores an alternative for those children who end up requiring follow-up surgery in cases where gross total resection is not achieved, or who experience recurrence.

This approach has proven to simplify second-look surgery for many other solid tumor types, the most comparable of which is now the standard of care for infants and children with ocular retinoblastoma (Rb).

This IA approach is perfectly suited for CPC, which parallels Rb in its anatomy and potentially shares similar biological mechanisms with respect to their chemosensitivity to drugs. The drug combination being used in the clinical trial has already been drug screened against a CPC cell line in collaboration with the NIH and showed much promise, and it proves to have a low toxicity profile when delivered intra-arterially.

Principal investigator Dr. Mark Souweidane leads this concentrated effort to bring therapeutic drug delivery alternatives to clinic. His renowned success with leveraging alternative drug delivery methods, compounded with his preclinical work to identify drug combinations for the CPC clinical trial, puts WCM in an optimal position to host this trial.

St. Baldrick's Foundation and American Cancer Society Award Collaborative Grant to Weill Cornell Medicine to Support "Liquid Biopsy for Longitudinal Monitoring in Diffuse Midline Glioma (DMG) Patients"



The esteemed Pilot Accelerator Award, a competitive grant awarded annually by the St. Baldrick's Foundation (SBF) and the American Cancer Society (ACS), has been awarded to support a novel research project spearheaded by the Children's Brain Tumor Project at Weill Cornell Medicine to help advance innovative translational pediatric cancer research conducted within the context of a clinical trial.

"Liquid Biopsy for Longitudinal Monitoring in Diffuse Midline Glioma Patients," is an innovative clinical trial led by principal investigators Dr. Mark Souweidane and Dr. Nadia Dahmane of Weill Cornell Medicine, in collaboration with Dr. Alex Miller at Memorial Sloan Kettering Cancer Center. The trial, which already received approval for enrollment from the Internal Review Board at Weill Cornell Medicine, involves the consistent monitoring of cerebral spinal fluid (CSF) from patients with diffuse midline gliomas (DMG) and tracking the patient's response to the administration of therapeutics. The Pilot Accelerator Award helps to bring this exciting clinical trial to fruition with a grant of \$240,000 over two years.

Traditional imaging-based methods often fail to produce an assessment of real-time treatment response in DMG patients, tumor biopsy is risky, and DMG pathology does not fully reflect the molecular profile of the tumor. Early assessment of treatment response via longitudinal monitoring may provide important information related to the value of therapeutic strategies and guide the selection of time-sensitive treatment.

This trial leverages longitudinal CSF sampling from DMG patients as a method to monitor their disease continuum from diagnosis to initial treatment to disease progression. Such analysis has high sensitivity and specificity for markers of DMG disease burden such as H3K27M mutation. Tumor-specific genetic alterations in liquid biopsy have the potential to be used as a surrogate marker of tumor burden, to monitor positive biological effects of treatment, predict disease progression, and adapt therapies in real time.

This clinical trial is intended to produce reliable tumor response data that may impact therapeutic options, optimize schema, and contribute toward time-sensitive treatment decisions.



Aiden Snyder

Hayes Tate

Clinical Trial for CPC funded in memory of Aiden and Hayes

The Snyder family and the Tate family share a devastating loss. Aiden Snyder was only 3 years old when he lost his life to a rare brain tumor called Choroid Plexus Carcinoma (CPC). The Tate family suffered the loss of Hayes to the same type of rare tumor when he was only 20 months old.

These families also share a determination to honor their beautiful boys by fundraising to change the outcomes for children in the future. Generous donations from the Aiden Snyder Foundation and Hayes Tough gave Dr. Souweidane the ability to bring this novel clinical trial to clinic, providing a new alternative option to families faced with a CPC diagnosis.

Recent Peer-Reviewed Publications from the CBTP Lab

COVER STORY IN MOLECULAR AND CELLULAR BIOLOGY

A paper from CBTP principal investigator Nadia Dahmane and her team was featured on the July 2021 cover of *Molecular and Cellular Biology*. The paper, "RP58 Represses Transcriptional Programs Linked to Nonneuronal Cell Identity and Glioblastoma Subtypes in Developing Neurons," explores the role of the transcription factor known as RP58 in managing the differentiation of neural cells. The team's investigation was highlighted in more detail in the fall 2020 newsletter alongside the announcement of Dahmane's corresponding RO1 grant. The paper shows for the first time that RP58 is a "master guardian" of the neuronal identity transcriptome, and that its function may be required to prevent the development of brain disease, including glioma progression.

NEURO-ONCOLOGY FEATURES REVIEW OF INTRACRANIAL GERM CELL TUMORS

Dr. Mark Souweidane recently collaborated with 24 esteemed investigators in the United States, Canada, France, Germany, and the United Kingdom to publish a new consensus statement on effective treatment strategies for intracranial germ cell tumors (iGCT). The paper, "Intracranial germ cell tumors in Adolescents and Young Adults: European and North American consensus review, current management and future development," was published in the November 1, 2021, issue of the journal *Neuro-oncology*.

Intracranial germ cell tumors primarily affect adolescents and young adults, with a much higher incidence in Asian countries. The goal of the consensus was to review and develop guidelines in North America and Europe based on effective strategies used in the West, which have resulted in survival rates of over 90 percent without the use of aggressive surgical intervention.

The new consensus statement, currently under review by the Society for Neuro-Oncology (SNO), European Association for Neuro-Oncology (EANO), and EUropean reference network for Rare Adult solid CANcers (Euracan), describes the successful strategy of treatment reduction to minimize long-term side effects.

Looking to the future, the collaborators stress the need for more clinical trials, more access to tissue samples for bench research, and a better understanding of the markers and mutations that characterize intracranial germ cell tumors.

RESULTS OF PHASE 1 TRIAL PUBLISHED IN JOURNAL OF NEUROSURGERY

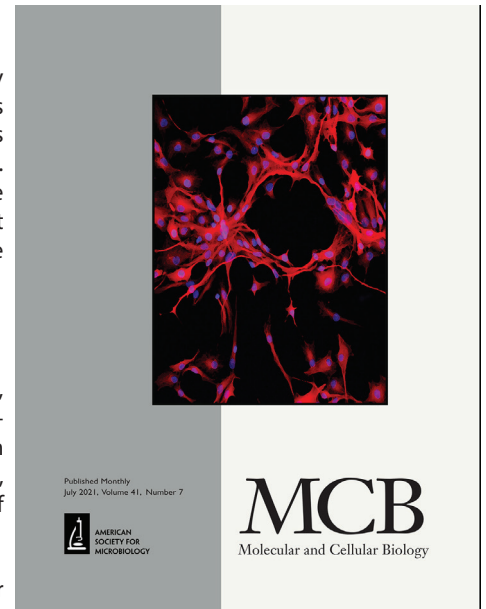
Designing new routes of administration to deliver targeted agents, antibodies, or classic chemotherapies to inoperable brain tumors is central to the mission of the Children's Brain Tumor Project. A major advance in these efforts was recently published from Dr. Greenfield's group in the August issue of *Journal of Neurosurgery: Pediatrics*. The paper reports on the results of the first-in-kind clinical trial of an intra-arterial approach to chemotherapy drug delivery to the brain of children. The paper, "Intra-Arterial Delivery of Bevacizumab and Cetuximab Utilizing Blood Brain Barrier Disruption in Children with High Grade Glioma and Diffuse Intrinsic Pontine Glioma: Results of a Phase I Trial," show intra-arterial delivery to be safe in pediatric patients. It also appeared to increase the average survival time for children with DIPG (patients in this study showed a mean survival rate of 17.3 months, compared with less than the one year typical for this terminal disease). As a result, Weill Cornell Medicine has recently approved expansion into a multi-center trial, designating the University of Miami as a second site for trial enrollment.

Intra-arterial delivery is a precise surgical approach that delivers chemotherapy drugs by threading delicate microcatheters to specific areas of the brain to increase drug concentration at the tumor site while reducing the potential for systemic side effects. This delivery method had not previously been tested in children with high-grade pediatric brain tumors including high-grade glioma and DIPG. Thirteen children were treated, and the method proved safe with no severe side effects. Central to this trial's success was its partnership with experienced neuro-interventional surgeons, in this case Dr. Jared Knopman at Weill Cornell Medicine, where the safety and efficacy of intra-arterial delivery for retinoblastoma was described by his colleague Dr. Pierre Gobin 20 years ago.

Expanding on Dr. Gobin's success with retinoblastoma, this new trial focused on delivering a combination of drugs — bevacizumab and cetuximab — through local arteries that directly feed a brain tumor. Prior to receiving the drugs to treat the tumor, patients were also given a dose of mannitol, a drug that opens the blood-brain barrier, to allow for the bevacizumab and cetuximab to better penetrate the tumor.

Working with Dr. Greenfield on this project was Dr. Heather McCrea, MD, a pediatric neurosurgeon at Nicklaus Children's Hospital/University of Miami. Dr. McCrea, who completed her residency at New York-Presbyterian/Weill Cornell Medical Center in 2016, worked on this project for several years with Dr. Greenfield, Associate Professor of Pediatric Neurosurgery and co-founder of the Children's Brain Tumor Project. Dr. McCrea trained under Dr. Greenfield and Dr. Mark Souweidane during her residency, then completed a fellowship in pediatric neurosurgery at Boston Children's Hospital before accepting her current faculty position at the University of Miami.

The exploration of alternative surgical approaches to drug delivery among pediatric brain tumor patients is foundational to the research underway at the Children's Brain Tumor Project, Weill Cornell Medicine. Strategies such as intra-arterial and convection-enhanced delivery allow for direct drug delivery to the tumor, effectively bypassing the blood brain barrier and reducing the systemic toxicity that is experienced with standard intravenous chemotherapy.





Matt Easley after completing the NYC Marathon.

Do Something Spotlight

On November 7, 2021, Matt Easley crossed the finish line at the New York City Marathon after running 26.2 miles. This year's race marked the 50th anniversary of the NYC marathon, which was exciting in itself, but Matt had even more reason to be proud.

Matt dedicated his run in memory of his friend, Elizabeth Minter, who died of a brain tumor in 2012. It was a very emotional accomplishment, and a gratifying achievement for Matt.

"Liz and I were good friends throughout our childhood in Bronxville, New York," says Matt. We graduated high school together in 2009 and shared a similar group of close friends over the years."

Through the partnership between Team Continuum and the Children's Brain Tumor Project/Elizabeth's Hope, Matt was able to raise more than \$4,000 to support our mission to cure pediatric brain tumors in her memory.

"Liz was a joy to be around and she was truly a special person. I always admired the way Liz was able to light up a room and make people around her laugh and have a fun time. Whether we were driving around listening to music, or hanging out with a group of friends, Liz's energy and personality always shined bright. She was an amazing person and I'm grateful for the opportunity to contribute towards her and her family's vision for Elizabeth's Hope!"

Congratulations, Matt! Thank you for this incredible accomplishment. Thank you for doing something.

If you are interested in running the NYC Marathon or Half Marathon in support of the lab, email info@childrensbraintumorproject.org.

It Takes a Village

From the desk of Lisa Lozzi Quinn

Motherhood is an extraordinary gift. The love between a mother and her children is immeasurable. The sheer thought of anything ever taking that dynamic away is not possible — until the evil of a cancer diagnosis for your child shockingly arrives out of the darkness.

At the age of eight, my daughter Keira was a beautiful, vibrant, and seemingly healthy child up until March 19, 2015. Out of nowhere, Keira woke up for school that morning with the left side of her face drooped as if she had a stroke. I immediately took her to the hospital. There are moments in time that we remember every detail of where we were or what we were doing when witnessing or hearing about something tragic. When the pediatric neurosurgeon reviewed the MRI with me, I felt like I was melting into the floor. Keira had an inoperable tumor in her brain stem. A monster called DIPG.

In my initial naïveté about pediatric brain tumors, I had no idea the enormity of what my little girl was up against. Sadly, we were forced to learn quickly. I now know the desperate need for monetary donations to go directly to pediatric brain tumor research. A mere 4% of government funding for cancer research goes to pediatrics and even worse, less than 1% goes to pediatric brain tumor research. Sadly, this is not a very well-known fact across our country. Our children are the future, and they deserve so much better than this unacceptable travesty of unfathomable proportions.

When Keira was sick, my local police department was instrumental in providing help and care as needed for her, even if it was just carrying Keira up and down stairs. After Keira's passing, they told me they were at the ready to join with me in having a fundraiser in my daughter's honor, to raise money for much-needed pediatric brain tumor research. My focus then turned to finding the proper avenue and doctors that needed funding. Doctors who want to make a difference in the lives of innocent children who are dealt an earth-shattering bad hand.

Thankfully, I found those doctors, Dr. Mark Souweidane and Dr. Jeffrey Greenfield at the Children's Brain Tumor Project. They have devoted themselves to finding a cure for pediatric brain tumors. They are striving for a cure. They don't want to see anymore children and their families go through this nightmare. 100% of all money donated to the Children's Brain Tumor Project goes directly to funding the doctors' much-needed research.

The first annual fundraising dinner to support pediatric brain tumor research, which the police department and I partnered on, was a big success. We look forward to a yearly event together in support of the Children's Brain Tumor Project. Our goal is to continue raising awareness and supporting these doctors who have dedicated their careers to make a difference for kids.



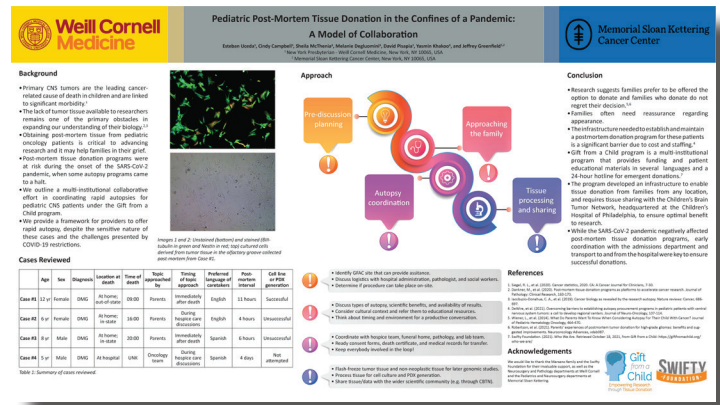
As a mom who lost my precious little girl to DIPG, the deadliest of all pediatric brain tumors, I ask you to please donate in support of research and to help raise awareness. The lives of future children literally depend on it! Spread the word!

Poster Accepted for Presentation at Society for Neuro-Oncology Annual Meeting

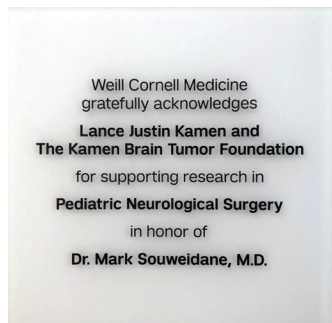
The Gift from a Child team at Weill Cornell Medicine worked closely with their partners at Memorial Sloan Kettering to share unique case studies on their efforts to coordinate post-mortem tissue donations over the past two years with the Society for Neuro-Oncology (SNO).

The abstract submission, "Pediatric Post-Mortem Tissue Donations in the Confining of a Pandemic: A Model of Collaboration," was accepted for poster presentation at SNO's esteemed annual conference, which took place November 18 - 21 at the Hynes Conference Center in Boston.

The SNO annual meeting is the world's largest neuro-oncology conference, attracting more than 2,600 researchers and clinician scientists from 40+ countries. Spanning all neuro-oncology disciplines, professions, and interests, the SNO annual meeting is a valuable platform for sharing knowledge and gaining insights on the future of neuro-oncology research and treatment.



Recognition for Extended Support from the Kamen Brain Tumor Foundation



A plaque has recently been installed in the CBTP lab to recognize the generosity of the Kamen Brain Tumor Foundation. Dr. Mazen Kamen and his wife, Jill, founded the Kamen Brain Tumor Foundation after losing their 19-year-old son, Lance, to a glioma in 2016. They have been generously supporting the lab ever since.

The Kamens recognize that traditional therapies for brain cancer are often unsuccessful, and they specifically support Dr. Souweidane's cutting-edge research into drug delivery alternatives as a potential game-changer in the way children with brain tumors are treated. The Kamen Brain Tumor Foundation is distinguished by its close contacts with medical experts in neuroscience and individuals in pharmaceutical and biotechnology companies who are working on novel research in the fields of immunotherapy and targeted gene therapy for this devastating disease. The goal of the foundation is to fund high-priority research studies that search for causes of pediatric gliomas and examine promising new treatments for these brain tumors. Their esteemed, multi-faceted scientific advisory board agree that the work underway at the Children's Brain Tumor Project is among those of high priority.

The CBTP Continues with Year-End Appeal to "Light up the Lab"

As 2021 comes to a close, the Children's Brain Tumor Project will continue the tradition of lighting up the lab in honor of children and young adults who have been impacted by a brain tumor diagnosis. We're asking all of our donors to honor the children who they hold so close to their hearts, and to help us light up the lab by making a year-end, tax-deductible contribution.

As in previous years, a designated website at lightupthelab.org will feature the stories of these children and enable donors to contribute to the year-end appeal. Building upon last year's campaign, we will be posting additional photos, videos, and blog posts throughout the month of December.

We light up the lab to honor the strength of families with a child in treatment, and the children who survived a difficult diagnosis, in celebration of many more holidays to come. The lightbulbs also represent the innovative ideas that are being explored in the lab as a result of donor support. Of course, we also light up the lab in memory of the children lost to this disease as a beautiful reminder that their lights shine on in our hearts.

All donors who make a year-end contribution will be provided with acknowledgement cards that can be given to friends and loved ones in lieu of, or in addition to, gifts. Thank you for helping to light the way toward scientific discovery.

Run the Half Marathon to Benefit the Lab!

The Children's Brain Tumor Project Foundation has been awarded a team in the United Airlines NYC half marathon on March 20, and the foundation hopes to have a team in the November marathon, as well. Charity registration for the half marathon begins December 8. If you or someone you know might be interested in running to support pediatric brain tumor research, you can request more information by emailing info@childrensbraintumorproject.org.



Dr. Greenfield Receives the Vision of Hope Award at Cristian Rivera Gala



The 12th Annual Cristian Rivera Foundation Celebrity Gala returned after a pause in 2020, and the Children's Brain Tumor Project was honored that Dr. Jeffrey Greenfield was presented with the esteemed Vision of Hope Award.

The black-tie affair was held on Wednesday, November 17, at Capitale (130 Bowery, New York City) from 6:00 to 11:00 P.M. Celebrities and NYC luminaries joined in celebrating Dr. Greenfield's contributions toward uncovering cures for children with brain tumors, including diffuse intrinsic pontine glioma (DIPG). All proceeds from this event will support DIPG research underway at Weill Cornell Medicine in memory of Cristian, who passed away from the deadly brain tumor in 2009. Cristian's father, John "Gungie" Rivera, started the foundation named for his son to help other families facing the diagnosis and to raise significant funding in support of research.

Cristian used to tell his father "I love you TEN!" That kind of extraordinary love is what inspired the theme for this year's gala. Visit cristianriverafoundation.org for more information and to make a donation in support of this inspiring event.

The Michels Family Endowed Professorship in Pediatric Neurological Surgery

Inspired by their teen daughter, Sophie, Tim and Barbara Michels recently pledged \$3 million to create an endowed professorship, the first in pediatric neurosurgery at Weill Cornell Medicine. The first faculty member to hold the endowed position will be Dr. Mark Souweidane, Professor of Neurological Surgery in Pediatrics at New York Presbyterian/Weill Cornell Medical Center.

At age 11, Sophie Michels was diagnosed with a rare brain tumor called choroid plexus carcinoma (CPC), a slow-growing cancer deep in the brain. Today she is a vibrant college junior who loves exercising, playing polo, and being with friends and family—all thanks to a remarkable endoscopic surgical technique pioneered by Dr. Souweidane.

"Her surgery and recovery were amazing," says Mr. Michels, owner and vice president of Michels Corporation, an international energy and infrastructure contractor based in Wisconsin.

"Sophie walked out of the hospital in 24 hours, versus the days she spent in the hospital following her other (brain) surgeries."

The endowment will allow Dr. Souweidane to further advance the field of minimally invasive surgical technologies, including endoscopic neurosurgery and the evolving repertoire of drug delivery alternatives.

Weill Cornell Medicine put the Michels family's generosity to work right away. The gift, which will fund the new professorship for generations to come, is already supporting research into Dr. Souweidane's drug delivery mechanisms.

"The generosity of the Michels family imparts an indelible mark on our program for children, who deserve the most innovative approaches to care for brain and spinal cord tumors," Dr. Souweidane says. "This extraordinary gift will fuel creative pediatric brain tumor research at Weill Cornell Medicine, impacting the lives of countless families around the world."

Ellie Ruby Foundation Supports the Research of Dr. Jeffrey Greenfield

In August, the Ellie Ruby Foundation announced a generous \$100,000 grant to the Children's Brain Tumor Project to support the research efforts of Dr. Jeffrey Greenfield, who is determined to find cures for rare pediatric brain cancers.

Ellie was diagnosed with pediatric glioblastoma when she was only 10 months old, and Dr. Greenfield has performed eight surgeries on Ellie since diagnosis day. Today she is doing well and her family created the Ellie Ruby Foundation to give back.

"It is not our right but our obligation and responsibility to share Ellie's story and raise awareness for pediatric cancer," said Ellie's mom, Brittany Cogan.



Brian, Ellie, and Brittany Cogan with Dr. Jeffrey Greenfield