Second Annual “No Laughing Matter” Event Raises $650,000

The second annual “No Laughing Matter” comedy night on October 22 raised more than $650,000 to support pediatric brain tumor research. The fundraising event was hosted at Carolines on Broadway by the Children’s Brain Tumor Family Foundation.

An all-star comedy line-up of talented men and women donated their time and talent to the laugh-out-loud fundraiser, 100% of the proceeds from which directly benefitted the Children’s Brain Tumor Project (CBTP) at Weill Cornell Medicine. The inaugural event in 2018 raised $450,000 to benefit the groundbreaking research of Dr. Mark Souweidane and Dr. Jeffrey Greenfield, co-founders of the CBTP. That total was surpassed by approximately 45% this year.

“We are so grateful to the Children’s Brain Tumor Family Foundation, our honorees, our dinner chair, the performers, the sponsors, and the volunteer staff who made this event such a tremendous success. We are so humbled to be the beneficiary of such a fun event in New York City, and we were completely blown away by the support,” said Dr. Mark Souweidane, co-founder of the CBTP and Director of Pediatric Neurosurgery at Weill Cornell Medicine.

The event included an emotional donation appeal for research funding. There were also several children and patient families in attendance to support the event, like ten-year-old Walker Lipton, who has been personally impacted by a pediatric brain tumor diagnosis. The most touching moments of the night included the resounding applause for these children at different times throughout the evening.

Liz Laugh Love
In addition to side-splitting comedy, the evening included an award presentation to Michael Minter and Emmie Hill, who helped their daughter, Elizabeth, with the launch of their nonprofit, Elizabeth’s Hope, before she passed away in 2012. Elizabeth’s Hope was the cornerstone for the creation of the Weill Cornell Medicine Children’s Brain Tumor Project.

The Lineup
So many wonderful comedians in the NYC area were willing to perform that the event ran into overtime. What a testament to the cause, and to the philanthropy that is demonstrated throughout this great city. Performers included: Mike Cannon, Rachel Feinstein, Nathan Habib, Jay Jurden, Jessica Kirson, Kevin McCaffery, Mark Normand, Domenick Pupa, and teenage comic Dylan Roche. The Master of Ceremonies for the second year was Michael Dean Ester.

“I was glad to be part of this show for the second year in a row. I have a child with heart disease and it has not been an easy road, so I find it very rewarding to lend my talent in support such a wonderful cause and help parents and children laugh out loud in the face of such a difficult diagnosis,” said comedian Jessica Kirson, whose recent special “Talking to Myself” was produced by Bill Burr and aired on Comedy Central December 6th.

The Research Appeal
This year, the research auction raised funds for a very specific project that will help to change the research landscape for pediatric brain tumors. Following the success of the event, and in collaboration with the Gift From A Child initiative, Weill Cornell Medicine has recently been named a regional “Center of Excellence” to assist families in making legacy donations at the time of their child’s passing. More information about this exciting announcement can be found on page 3.

Our Sponsors
A fundraiser of this magnitude relies on sponsors to ensure financial success. For the second year in a row, the CBTP is grateful to Ms. Tory Burch for being our headlining sponsor. Her support has been unwavering.

Junior’s Cheesecake also provided dessert for the second year in a row, and Juice Press introduced attendees to their fantastic popcorn. The event was also supported by And That SWAG, Archipelago, Ahava, Big Hungry Bear Productions, Boombas, Burt’s Bees, Dr. Hauscha, Eylure, Heidi Green Photography, John Frieda, Sally Hansen, and Smashbox.
The Children’s Brain Tumor Project Increases its ‘Brain Power’

The Children’s Brain Tumor Project saw an explosion of growth in 2019, and as we embark on new research initiatives we need to expand our team. Dr. Mark Souweidane and Dr. Jeffrey Greenfield are thrilled to welcome six more outstanding team members who are already working hard toward new discoveries.

**POST-DOCTORAL RESEARCHERS**

**MYLENE BRANCHTEIN**

Mylene is new to Dr. Greenfield’s team after recently graduating from the University Libre of Brussels with a PhD in immunology. Mylene has been focused on regulatory T cells to understand their heterogeneity and their suppressive mechanisms in order to use them efficiently in therapy. In her new role at Weill Cornell Medicine, she is working with Dr. Greenfield on immunophenotyping of pediatric brain cancers and the effects of radiotherapy on these cancers with special focus on the immune cells in these cancers.

Prior to completing her PhD, Mylene obtained her master’s degree at Francois Rabelais University in Tours and her bachelor’s degree in cellular biology at Diderot University.

**BRICE MARTIN COUILLAUD**

Brice Martin is a new post-doc on Dr. Souweidane’s team, having just graduated from the University of Paris Descartes with a PhD in pharmacology. Throughout his studies, Brice developed nanoparticles (etoposide nanocrystal) as a new drug delivery system to treat carcinoma colon cancer in mouse models. Brice is now working on new strategies for delivering drugs to pediatric brain cancers with the goal of achieving a better therapeutic outcome than conventional therapies. Much of his focus will address the issues of pharmacodynamics and pharmacokinetics in order to improve the effectiveness of existing drug therapies.

In addition to completing his PhD at Descartes University, Brice received his master’s degree at School Polytechnic at University of Pierre and Marie Curie, his bachelor’s degree in chemistry at Diderot University, and he completed preparatory class at the Orsay University.

**DAISY LIN**

Daisy Lin just recently joined the team as a designated Clinical Trials Program Manager. Prior to joining Weill Cornell Medicine, Daisy was an Assistant Professor/Principal Investigator at the State University of New York, Downstate Medical Center, where she focused on the effects of anesthetic exposure on the developing brain. She is now working closely with Dr. Souweidane and Dr. Greenfield to design protocols for early phase clinical trials for children with central nervous system tumors. In addition, she will ensure the success and continuation of clinical trials by writing and applying for grants, serving as a liaison with cooperative groups, structuring agreements with industry, and interfacing with the internal review board (IRB).

Before starting her research career at Downstate Medical Center, Daisy was trained as a post-doctoral fellow at Columbia University Medical Center, where she investigated the role of epigenetic mechanism in neuropsychiatric disorders. Daisy obtained her Ph.D in molecular-cellular biology from SUNY Downstate.

**OTHER NEW ADDITIONS TO THE SCIENTIFIC RESEARCH TEAM**

**MAHABUB MARAJ ALAM**

Joining the team from upstate New York, Maraj Alam is a research technician working with the Dahmane and Greenfield research groups. Maraj received his bachelor’s degree from Cornell University, then spent two years working on brain injury models at Albany Medical College in Albany, New York. Now at Weill Cornell Medicine, Maraj is working to find the molecular binding partners of proteins that play a role in the development and progression of brain tumors.

True to his upstate roots, Maraj enjoys winter sports and activities, and he isn’t a big fan of the subway.

**TATYANA GONGORA**

Tatyana joined the Dahmane and Greenfield research teams in the summer of 2019. She is a technician focused on ensuring our lab has the best mouse models to work with. She manages the colony and handles surgeries/injections, watching tumor growth closely and making adjustments to her methods in order to create mouse models that closely mimic the behavior of human brain tumors.

Tatyana is a recent graduate of Bucknell University, where she received her bachelor’s degree in biology.

**ESTEBAN UCEDA**

Esteban is a new research technician working on Dr. Greenfield’s team. His role is to serve as a liaison between precision medicine initiatives and the research performed in the CBTP lab. Much of his focus is on developing patient-derived cultures and performing high-throughput drug screening to discover new drugs that can be used for treatment.

Like Dr. Greenfield, Esteban is a graduate of Amherst College with a bachelor’s degree in neuroscience.

---

**Save the Dates**

**FEBRUARY 5** - Dancing with the ChadTough Stars, The Chad Tough Foundation (Ann Arbor, MI)

**FEBRUARY 26** - Register to run for the Children’s Brain Tumor Family Foundation in the 2020 NYC Marathon

**MARCH 7** - The Greatest Gala, Ty Louis Campbell Foundation (Greenwich, CT)

**MARCH 7** - Casino Night, Brooke Healey Foundation (Somerset, NJ)

**APRIL 25** - Party with a Purpose, McKenna Claire Foundation (Huntington Beach, CA)

**MAY 1** - Annual CBTP Family Council Meeting (New York, NY)

**MAY 2 or MAY 30** - Cristian Rivera Foundation 5K (New York, NY)

**JUNE 13** - Head for the Cure 5K (Bronxville, NY) subject to change

---

$80,000

Congratulations to the CBTF Marathon Team!
Gift from a Child Names the CBTP a Center of Excellence

The Gift from a Child Initiative, primarily funded by family-founded nonprofit The Swifty Foundation, has designated the Children's Brain Tumor Project at Weill Cornell Medicine to serve as its fifth named “Center of Excellence” in the US. The initiative will enable the expansion of the pre-existing legacy donation program at the lab by funding an individual tissue navigator position over the next three years. This individual will educate patients, families, and staff about the value of tissue donation, assist patients and families with the logistics related to making such a donation at the time of a child's death, and coordinate tissue donations from other local and regional centers that don’t have formal donation programs in place.

Tragically, thousands of children die from brain tumors every year. Researchers and physicians do not know why the treatments do not work for these children. Until researchers are able to study the brain tissue of children who do not survive their cancer, it will remain a mystery why these children do not survive their disease. More so, it leaves the clinical team in the dark when treating new patients facing similar outcomes.

The Gift from a Child program addresses this need by facilitating the collection of a critical mass of brain tumor tissue samples to inform scientists on how tumors behave and how to prevent them from forming. Understanding more about each unique tumor type allows doctors to prescribe specific treatments for each individual child. This helps to minimize the harmful side effects of many treatments that are currently available, and increases the quality of life for children diagnosed with these diseases.

Due to the nature of the post-mortem program, referrals are made at all hours of the day and night. Without collaborative systems like this in place, and dedicated individuals serving as tissue navigators at each regional site, these precious donations are not be possible.

Centers of Excellence are regional autopsy sites designated to coordinate, process, store, and share post-mortem materials that enable research across institutions and increase information sharing. The collaborative of these centers, including Stanford Medicine, Children's Hospital of Philadelphia, Children's National Hospital, Lurie Children’s Hospital, and now Weill Cornell Medicine, delivers best practices in family/patient care and donation coordination.

The team at the Children's Brain Tumor Project is grateful for the support of Gift from a Child, and for the strength of all participating families who are faced with the difficult reality that no child or family should ever have to face.
Running for Joey

On February 7, 2016, my 8-year-old son Joey gained his wings after a 14-month battle with Glioblastoma Multiforme, a deadly brain cancer. Joey was smart, kind, extremely funny, incredibly athletic, and just the best kid all around. His beautiful blue eyes and bright smile lit up every room, and he was remembered everywhere he went.

Months later, while he was home on hospice care, I promised him that I would do everything I could to help find a cure for this disease—that I didn’t want to see any more families in the position that we were in.

On November 3, 2019, I ran the New York City Marathon with the Children’s Brain Tumor Family Foundation in honor of my angel Joey. In honor of Sean, Caitlin, Allie, Juliana, Daryn, Ty, and all the children who have battled brain cancer. It was hard, emotional, rewarding, and so surreal! I had the best supporters of family and friends along the entire route and I am so thankful for each one of them cheering me on and giving me motivation to keep on going.

At Mile 16 I saw my biggest supporters. It was so great to see the smiling faces of my husband, Joe, and my son Robbie. They stood by me every step of the way during training, and they cheered me on and again at Mile 25. I have the best video of Robbie saying, “Let’s go Mommy!” that I will cherish forever.

Some days I still can’t believe that I crossed that finish line! As I ran through the streets of New York City, complete strangers were screaming, “Go Joey’s Mom! You got this Joey’s Mom! Joey’s Mom you’re awesome!” It was a feeling that I can’t really describe but hearing his name for the 6.5 hours it took me to finish warmed my heart so much. I love you and I miss you, Joey.

Jennifer Clayton

CBTP “Do Something” Spotlight

Uniting a Community

Not unlike many members of the CBTP Family Council, Kathy and Joe Arabia had never heard the words “gliomatosis cerebri” until their daughter, Anna Yan Ji, was diagnosed with the terminal brain tumor in 2009.

“...we could not find anyone else who had been diagnosed with the same disease. As many do, we reached out to the internet and social media,” said Kathy. “At the time there was no research being done on this disease and no existing online community where gliomatosis cerebri families could connect. After three years they were so happy to connect with Emmie Hill and Elizabeth’s Hope.”

A few more families connected shortly thereafter and the dedicated Facebook group, The GC Connection, was created. The Arabias were instrumental in connecting with physicians and researchers to advocate for and support the first GC International Conference in 2015. This conference helped connect GC families, researchers, and physicians in a united effort for the first time, creating avenues for sharing information around the world.

“At the time, we found that there was very little information available to families like ours,” said Kathy. “We are pleased to say this is no longer the case.”

Anna was only 16 years old when she became an angel on Valentine’s Day 2013, filled with love and grace.

Losing Anna left Kathy and Joe with a need to do something more. They launched the AYJ Fund with the purpose of bringing smiles to kids with cancer, connecting kids with school and friends through technology, and supporting research for gliomatosis cerebri and other brain cancers.

In addition to running the AYJ Fund and hosting fundraisers throughout the year, they are extremely active with GC Global, both in planning the bi-annual conference and in keeping the Facebook page up to date. Kathy can be found adding new members, posting news items, or sharing awareness activities at all hours of the day.

Somehow the Arabias also find time to deliver on their annual holiday backpack program. This holiday season they have been busy sending packages out to hundreds of children in treatment for brain tumors. The Children’s Brain Tumor Project has helped distribute these bags full of goodies to children in the clinic every holiday season.

The AYJ Fund enables Kathy and Joe to continue honoring Anna’s love of helping others. It is their way of doing something, and it is making an impact in the lives of children with brain cancer.
EXPANDING THE TEAM
In research, the greatest investments are made in the people doing the work. We can't make discoveries in the lab without having brain power at the bench, and we can't bring those discoveries to clinic without a motivated translational team. In 2019, the Children's Brain Tumor Project welcomed eight talented new researchers with various levels of expertise to help move our research forward. The CBTP lab has become over-loaded with activity, and the space is getting crowded. This is certainly a good problem to have when your goal is to increase the speed at which scientific breakthroughs are made!

GROWING THE PRECISION MEDICINE INITIATIVE
Dr. Jeffrey Greenfield was awarded $1.08 million in support of his precision medicine initiative. The generous gift from the Patrick Bayly Marsano Foundation is enabling a tremendous increase in defining individualized cancer therapies for children with rare and inoperable pediatric brain tumors. The two-year initiative is funding the expansion of the lab’s cellular and molecular precision medicine approach to pediatric brain tumor therapy, with a goal of establishing safe and curative patient-specific therapies for devastating pediatric brain tumors. Clinicians, computational biologists, neuroscientists, immunologists, biochemists, and stem cell biologists, have begun working together to conduct next-generation sequencing on every pediatric brain tumor resected at the Weill Cornell Medical Center—an anticipated 50 to 80 children. They will identify new mutations specific to the cell population and to the patient, and create cell repositories and mouse models to test different targeted therapeutics. Following a comprehensive review of the genetics, cell biology, and pharmacogenomics of patient-specific tumors, a customized therapeutic regimen will be determined for each patient.

ADVANCING TRANSLATIONAL RESEARCH
Translational research is often referred to as the process of applying findings from the laboratory to practices in a clinical setting. Our team recently finalized an agreement with Macrogenics, a biopharmaceutical company, to provide us with a promising antibody drug conjugate to target an antigen commonly found in pediatric brain tumors. The study will help to evaluate the antitumor efficacy and pharmacokinetics of the drug, informing our design of a possible FDA approved clinical trial for children that uses convection-enhanced delivery. This agreement with Macrogenics demonstrates our reputation in the research community and among industry representatives who want to partner with us.

MAKING CONTINUED INROADS IN COLLABORATIVE EFFORTS
In the December newsletter we share that the Children's Brain Tumor Project has recently been named a regional “Center of Excellence” by the Gift from a Child initiative. This is a game-changer not only for the CBTP, where it will increase the amount of rare tumor tissue we have access to through rapid autopsy, but also for our collaborating institutions through the Children's Brain Tumor Tissue Consortium, with whom we will share access to that tissue.

Similarly, the DIPG Collaborative has accepted the CBTP protocol for participating in the DIPG registry, another vehicle for sharing detailed information specific to DIPG tumor tissue across a multitude of participating institutions.

In addition, Dr. Mark Souweidane and Dr. Jeffrey Greenfield recently received faculty appointments to the pediatric neurosurgery team at Columbia Medical Center, which means they are able to bring alternative drug delivery methods including convection-enhanced delivery and intra-arterial delivery to even more patients outside of their pre-existing practices at Weill Cornell Medicine, Memorial Sloan Kettering and New York-Presbyterian.

APPLYING FOR GOVERNMENT FUNDING
An RO1 research grant from the National Institutes of Health (NIH) is an award made to support a specific project representing the investigator’s specific interest and competencies. RO1 grants are very competitive with less than 1/5 of all research applicants receiving funding (~20%).* A percentile ranks each application relative to the other applications reviewed within a study section. The CBTP RO1 applications in 2019 received rankings as high as the top eighth percentile, which means additional government funding will likely be awarded to help expand our initiatives even further in 2020, but we still have to wait to receive official word.

*approximate estimate that varies annually depending on government appropriations.

PUBLISHING PEER-REVIEWED ARTICLES
Listed on the back is a collection of published research papers to which members of the CBTP have contributed in 2019. Peer-reviewed publications provide proof of validation for completed research because they subject the authors' scholarly work to a very high level of scrutiny from other experts in the field who evaluate whether an article is suitable for publication. The sheer volume of published work is a testament to the hard work and dedication of our lab team.
Combined targeting of PI3K and MEK effector pathways via CED for DIPG therapy

AUTHORS: Raymond Chang, Umberto Tosi, Julia Voronina, Oluwaseyi Adeuyan, Linda Y Wu, Melanie E Schweitzer, David J Pisapia, Oren J Becher, Mark M Souweidane, Uday B Maachani

ABSTRACT: The manuscript showed the results of a comprehensive study of midline gliomas including diffuse intrinsic pontine gliomas (DIPG), and the relationship between amplified phosphatidylinositol 3-kinase (PI3K), mitogen-activated protein kinase (MEK), and the development of these particular tumor types. Studies have implied that amplifications in the PI3K signaling pathway may result in tumorigenesis, and that the activation of parallel pathways (e.g., mitogen-activated protein kinase (MEK)) may be causing the resistance to PI3K inhibition that has been observed in the clinic. An accurate understanding of this relationship could open doors to potential new treatment options for children with DIPG and other midline gliomas in the future.

Real-Time, in vivo correlation of molecular structure with drug distribution in the brain striatum following convection-enhanced delivery


ABSTRACT: In this study, researchers modified an existing therapeutic commonly used for Leukemia (dasatinib) to monitor the movement of the drug in real time via PET imaging. The team created a panel of significantly improved dasatinib analogues, which can now be observed as they move across the brain and leave the site of injection over time. In other words, the research team was able to watch the dissemination of the drug in vivo when delivered via CED, and tweak the analogues with the goal of seeing concentrated drug present at the tumor site, extended half-life, and little movement throughout the rest of the body. The findings from this study are of paramount importance as they relate to the team's parallel work studying and fine-tuning the use of convection-enhanced delivery (CED) to treat DIPG, a technique pioneered by Dr. Souweidane using a cannula to deliver drugs directly into the tumor tissue in order to bypass the blood-brain barrier and reduce systemic toxicity.

ADDITIONAL PUBLICATIONS:

The intersect of neurosurgery with diffuse intrinsic pontine glioma.

AUTHORS: Kuzan-Fischer CM, Souweidane MM.

Phase II Trial of Response-Based Radiation Therapy for Patients With Localized CNS Nongerminomatous Germ Cell Tumors: COG.


Phase II Study of Nonmetastatic Desmoplastic Medulloblastoma in Children Younger Than 4 Years of Age: COG.


Integrative Molecular Analysis of Patients With Metastatic Cancer.

AUTHORS: Miller AM, Shah RH, Pentsova EI, Pourmaleki M, Briggs S, Faltas BM, DeAngelis LM, Young RJ, Berger MF, Mellinghoff IK.


Endonasal surgery for suprasellar germ cell tumors: two cases and review of the literature.


Transcriptome signatures associated with meningioma progression.


Developing a 3D composite training model for cranial remodeling.


Transcriptome signatures associated with meningioma progression.


Tracking tumour evolution in glioma through liquid biopsies of cerebrospinal fluid.