

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

June 2020

Despite Closed (Laboratory) Doors – Research Moves Forward



Backdrop of the Children's Brain Tumor Project Lab, Weill Cornell Medicine

Starting in March, teams of cancer researchers across the country and around the world feared their work would come to a screeching halt when shutdowns were ordered and research priorities shifted to address the emerging global health threat of COVID-19. Instead of allowing the crisis to pause progress, the team at the Children's Brain Tumor Project (CBTP) sprang into action to find ways to maintain momentum on pediatric brain cancer research while immunologists and virologists committed to addressing the pandemic. Lives are at stake in both cases, and the research underway at the CBTP remains equally essential.

"As a community of scientists, we reacted quickly and strategically to ensure that the lab shutdowns wouldn't have a negative impact on the great work that was already underway in our lab, or the broader cancer research landscape," said Dr. Jeffrey Greenfield, Vice Chairman of Academic Affairs and Associate Professor, Department of Neurological Surgery at Weill Cornell.

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From the Desk of... Julie Balay Chase's Gift

Car ride conversations with my little boys Ryder and Chase were always fascinating and ranged from ultimate silliness to more sobering topics like death...perhaps prompted by passing a graveyard.

Of course, it would always be my death we spoke about. Years in the future I would get old and die, when they were grown men with their own lives and families. Would I want to take up space in the ground or be cremated so they could each carry me around and sprinkle me in cool places? "Definitely the latter for me," I would profess. "Me too!" they both agreed. "What about my organs? What if someone could use my heart or my kidneys? What do you guys think? Would you want to give someone a part of you?"

My Chase, who was perhaps a ripe six years old, unequivocally with the practical wisdom of an elderly man plainly answered, "Of course, why would I want to keep them? I won't need them anymore."

I occasionally had similar conversations with them over the years, never once suspecting that it would be me making that decision when Chase died at the age of 10, a short 15 months after being diagnosed with a rare glioma. I am eternally grateful today that before brain cancer entered our lives, I already knew what my sweet son would want in the event of his passing. Although unlike live organ donation, his tissue was not directly used to save a life, the truth is that his gift will save many more than just one...

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Missing Diversity in Brain Tumor Trials

Neuro-Oncology Advances, June 2020

The team at the Children's Brain Tumor Project recently contributed to a scientific study that explored ethnic and racial representation in brain tumor trials and found that despite increasing representation in the American population, minorities are under-represented in brain tumor clinical trials.

In addition, the study revealed that—although numerous legal requirements and ethical mandates are in place—race-based information is remarkably absent from a majority of the published results from brain tumor trials conducted over the last decade. In fact, 70% of brain tumor trials didn't even include race-based patient demographics to allow for stratifying data for analysis.

The aim of this study was to quantify and map ethnic and racial representation in brain tumor trials and to examine the potential gaps in trial recruitment. The team analyzed trials registered with clinicaltrials.gov between July 1, 2005, and November 11, 2017, using a combination of PubMed/Medline and Google Scholar to find publications associated with those trials, including patient demographic information (where available).

After reviewing 471 trials and applying expertise in bioinformatics to analyze the data, the findings were truly eye-opening with regard to the lack of diversity recorded in brain tumor trials. Not only did the trials have significant gaps in representation, those gaps persisted even

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Make a donation today at ChildrensBrainTumorProject.org

Virtual Fundraising, Research Updates and Education



Dr. Stieg and Dr. Greenfield with Dakkar team in 2019, and during an online lecture in 2020

Dr. Greenfield's Online Lecture with Neurosurgeons in Africa

On June 10, Dr. Greenfield spent the afternoon presenting a virtual lecture on minimally invasive surgery for pediatric brain tumors to more than 50 neurosurgeons in Africa who he previously met while in Dakkar. It was a wonderful example of how technology can bring the world closer despite travel being cancelled.

The 2020 Family Council Meeting

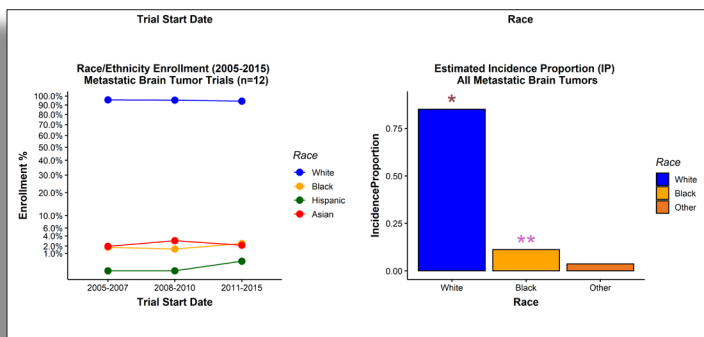
The annual Children's Brain Tumor Project Family Council Meeting is a day where we welcome patient families and CBTP supporters to meet the team and hear about our most recent research. On June 2, our Principal Investigators hosted a productive virtual family meeting that was attended by more than 70 families, nonprofit organizations, and researchers who are interested in making advances in neuroscience.

Missing Diversity in Brain Tumor Trials (continued from page 1)

in dense multicultural centers like New York City.

We know that there are often important variables to consider across different genders, ethnicities, and races when studying any particular disease. Trials should be designed to require appropriate diversity to accurately represent the patient population, and ensure those trials are easily accessible to a diverse population.

This information is important to consider when drawing conclusions from existing published results, in addition to designing present-day clinical trials. Diversity is essential for scientific accuracy, and the proper planning and reporting of race in clinical trials is needed to account for differences in presentation, treatment and response. The Children's Brain Tumor Project is proud to shine a light on this issue and advocate for changes in trial design as a result.



Dr. Souweidaine participating in ChadTough Live with Dr. Carl Koschmann, Michigan Medicine, and host Jay Feely, CBS Sports

Virtual Check Presentation from the CBTF

On Friday, May 1, the Children's Brain Tumor Project (CBTP) received a much needed, much appreciated gift from the Children's Brain Tumor Family Foundation. In a live-streamed online meeting, the foundation presented a check for \$525,000 to fund life-saving pediatric brain tumor research at Weill Cornell Medicine.

Chad Tough Live

On May 17, Dr. Mark Souweidane was invited to present during *Chad Tough Live: Tougher Together*—a virtual event featuring stories from some of the foundation's biggest supporters, doctors, and families affected by DIPG. The recording is available at chadtough.org/live.

Fusions Involving BCOP and CREBBP are Rare Events in Infiltrating Glioma *Acta Neuropathologica Comms, June 2020*

Dr. Jeffrey Greenfield recently contributed to the publication of a peer-reviewed article describing a rare BCOR-CREBBP fusion in a pediatric patient with a high-grade infiltrating astrocytoma.

Sequencing analysis of 686 primary CNS tumor cases (adult and pediatric), led the team to team identify an additional case demonstrating a BCOR fusion to a paralog of CREBBP, namely EP300. In addition, they detected three additional fusions involving either BCOR or CREBBP.

These findings add to existing literature implicating BCOR as having a potential driving role in CNS tumors. However, given that the BCOR-CREBBP fusion was not found to be recurrent and may represent a stochastic event, further screening and functional studies are warranted to further define the oncogenic potential of BCOR and related fusions in infiltrating gliomas.

Best Neurosurgeons in NY 2020

The Weill Cornell Medicine Brain and Spine Center is filled with specialists who routinely appear on lists of Top Doctors, Best Doctors, and SuperDoctors, and 2020 is no exception. This year's roster of Castle Connolly Top Doctors includes a record 13 members of our faculty at Weill Cornell Medicine, including Dr. Jeffrey Greenfield and Dr. Mark Souweidane. We are so proud of our neurosurgeons and their staff who make our Brain and Spine Center the outstanding center that it is.

Despite Closed Doors – Research Moves Forward (continued)

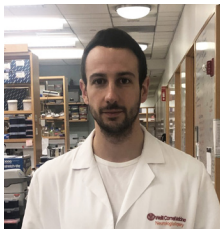


Mylene Branchtein

At the onset, the team reacted quickly to develop emergency protocols and an on-site rotation to care for the live specimens in the lab, while testing new team software and remote-working tools to maintain connectivity. Lab meetings were hosted via Zoom at least twice a week, and these online platforms triggered scientific brainstorming with explosive ideas. Now the team is returning to the bench inspired to apply those ideas, a sentiment that is echoed in many of their personal comments below.

Inspiring New Ideas

"When I heard about the lab shutdown, I wasn't sure what I could do from home during those long weeks. I soon realized it gave me time to read and learn a lot about my research project, time that I usually don't have. And now I am returning to the lab with so many ideas for new experiments! I really think this time has been so beneficial for all of us." *Mylene Branchtein*



Brice Martin

Maintaining Momentum, Because Cancer Doesn't Stop During a Shutdown

"When someone has cancer, the whole family and everyone who loves them does, too. It may feel like COVID-19 has brought everything to a halt, but obviously that doesn't hold true for cancer. Upon news of the shutdown, I was greatly concerned about losing momentum on the ongoing cancer research underway at the lab, and equally concerned about the patients and their families who might struggle to receive treatment during these unprecedented times. Thankfully, the fast action of my team and the commitment to patient care at Weill Cornell has eased these fears and allowed me to continue with my dedicated work toward curing rare and often incurable brain tumors." *Brice Martin*



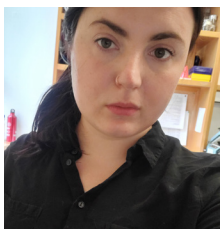
Esteban Uceda

Finding Creative Ways to Work from Home

"I think we all saw the shutdown coming, especially towards the end of February when it was becoming clear that the situation in the US was likely to progress rapidly (as it did). We worked extra hard at making sure all non-essential activities were stopped swiftly and finishing off ongoing experiments and collecting lots of data we could analyze from home. My work shifted to include a lot of research and reading what other labs are doing, as well as meeting with our collaborators to ensure that once research activities resumed, we could restart experiments quickly." *Esteban Uceda*

Thrilled to Reopen

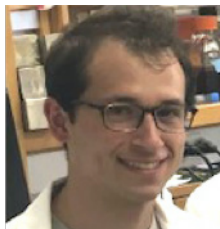
"I was sad and anxious to hear about the lab shutdown. Some of the most interesting experiments that we were planning for a long time, which involved breeding mice with a certain genotype, had to be put on hold. We had to make a lot of adjustments to the care of our mice colony. I was so worried about how much it would take to restart normal lab flow upon reopening that I started to prepare by coming in a few days a week to maintain the lab and keep up momentum. I'm happy to report it's been a very smooth transition to return to the lab." *Tatyana Gongora*



Tatyana Gongora

Rising to the Challenge

"Since the beginning of the coronavirus crisis, everyone on the lab team has been working together to get things done. Lab members have coordinated with each other to make sure that each person who goes into the lab can accomplish other people's tasks, allowing us to continue to be compliant with social distancing regulations." *James Agolia*



James Agolia

Maintaining Connectivity with the Team

"When everything started, I worried about how I might keep my research going when I'm so far from the bench. However, we found ways to remain extremely connected with the rest of the team, and soon I realized that being home has some good potential. As a matter of fact, during these days I had the time to analyze my most recent data, to finalize some of my projects, to study, and to plan in detail what to do next for my experiments. I cannot wait to start working on my new ideas!" *Carolina Cocito*



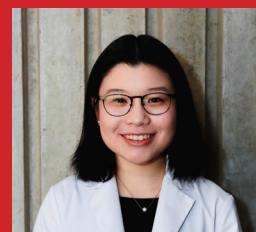
Carolina Cocito

Optimistic About the Fast-Paced Return

"Though the shutdown is undoubtedly a setback, I believe that the team at the Children's Brain Tumor Project, along with the broader research community, will rise from it with renewed strength. It may not be possible to come out of this pause right where we left off, but we can come back from a new place. As we take time now to think, reflect, and plan, a wave of energy builds. And as the gate is opening, we are on the other side sprinting back in, ready to tackle cancer anew." *Rachel Yan*

Rachel Yan Awarded Funding from Alex's Lemonade Stand

The Alex's Lemonade Stand Foundation (ALSF) Pediatric Oncology Student Training (POST) Program has awarded funding to Rachel Yan, a student working under the mentorship of Dr. Mark Souweidane at the CBTP lab. The program is designed for students interested in pediatric oncology research. Rachel is currently a second-year medical student at Rutgers Robert Wood Johnson Medical School. She is returning to the Children's Brain Tumor Project for a research year after spending the previous summer with us. She graduated from the University of Wisconsin-Madison in 2018 with a degree in Genetics, while working on research in cancer genetics, targeted therapy, and immunotherapy at UW-Madison and Brigham and Women's Hospital.



Rachel Yan

From the Desk of Julie Balay

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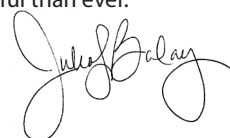
The actual process of Chase's gift was seamless, which was a vital necessity at such a brutal time when there is no room for much more than taking your next breath. A few papers, a heartfelt promise that

our boy would be lovingly cared for, and immense gratitude from the team are all I recall. Then, in the saddest months that followed his death, I was able to actually see Chase's alien green dyed cells (his favorite color!) with my own eyes! Oh, what a feeling to witness that a part of him still lived here, with all of us. Bittersweet tears flowed.

The depth and permanency of the pain experienced in child loss is something indescribable. I will never get to stand proudly at his graduation or brag to everyone about how he's living his dream as a professional artist. But I can still be proud! I can still brag away! Because you see, my son's cell lines live in a laboratory where they are teaching these dedicated doctors and scientists what they need to know to eradicate this evil disease. On the day we see a cure, likely in my lifetime so I am told, I can brag that my son Chase was part of that.

I don't know if there could be a prouder parent moment than having a child who so selflessly saved the lives of others. If the story of Chase's gift can inspire another family to do the same, then his legacy will grow stronger and more meaningful than ever.

We miss you every day, Chasey!!



CBTP "Do Something" Spotlight Applying Her Love for Science to Help Children

Having dreamed of being a neurosurgeon since she was just five-years-old, Nikki Lyons (27 years) always found the brain to be fascinating. However, she had never anticipated her career interest in the field of cancer research.

"My stepdad had battled leukemia and lymphoma for years. Watching someone undergo treatment for cancer was traumatizing, so my initial educational pursuits were focused elsewhere," said Lyons.

But in 2015, Nikki was introduced to Al and Patti Gustafson, who lost their son Michael to a brain tumor called medulloblastoma. She was so inspired by their story and immediately wanted to help. Through the Swifty Foundation, a nonprofit they founded in Michael's memory, the Gustafsons created a program that would better enable bereaved families to donate tumor tissue via rapid autopsy, and to share that tissue for the benefit of pediatric brain tumor research.

"Everything they were doing with the *Gift from a Child* donation program really hit a chord with me. I had a kidney transplant when I was 23 years old. That organ donation gave me life, and these brave families who opt to donate their child's tumor tissue after losing them to a brain tumor are giving that same potential gift of life to children diagnosed with brain tumors in the future," she said.

The mission of the Swifty Foundation to develop a rapid autopsy donation program was something Nikki knew she wanted to be involved with, and it inspired her to explore various ways she can contribute to curing pediatric brain tumors by immersing herself in the research.

Thus began her thoughtful contributions to help advance the science that may one day lead to a cure for children with pediatric brain tumors. While pursuing her undergrad at the University of Illinois, Nikki had the opportunity to spend a semester in the pediatric brain tumor lab at Lurie Children's Hospital where she studied checkpoint inhibitors in various types of brain tumors. Since moving to New York to attend Columbia University, Nikki also spent the summer working at the Children's Brain Tumor Tissue Consortium in Philadelphia — a collaborative, multi-institutional research initiative that Weill Cornell has participated in since 2016.

"Having been a young adult when I suffered my own medical trauma, I find myself gravitating toward pediatrics and developmental biology, which is fascinating to me," said Lyons. "I also have a very strong interest in bettering the experiences of adolescents and young adults who suffer chronic medical conditions. I know first-hand how isolating it can be, and I hope to change that culture, as well."



Nikki Lyons



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