



Dr. Jeffrey Greenfield and Dr. Mark Souweidane, co-directors of the Weill Cornell Medicine Children's Brain Tumor Project

From the Desk of...
Brenda Ríes

Waiting. I feel like that's all we have been doing this year—waiting. On February 2, 2017, a routine MRI showed a new spot in my son's brain. It was exactly five years to the day from the very first symptom he had, symptoms that days later would lead to the discovery of a 5-centimeter tumor in his brain. After three surgeries to remove most of the tumor, and almost five years of stable scans, how could this be happening again? Isn't "five years" the magic number when everything is supposed to be okay now?

We were devastated all over again; this relapse hurt almost as bad as his original diagnosis did. We caught it early, though, and the new spot was small, so doctors felt there was no rush to take action. Maybe it wasn't a new tumor—there was a chance it might be something else. The recommendation was to wait, scan again in three months, and see what happened. So, as hard as it was, we waited and we tried to forget and just live a normal life, but really there is no "normal" anymore and there is no forgetting—it's there every day and it's always on your mind. What is happening inside that cute little head? Is the tumor growing? Should we be doing something more? What will we do next? As a parent you feel completely helpless, and so you research, you read, and you wait.



May finally came and it was time for that next scan, and once again it was not the news we had hoped to get. This new spot had grown and needed to come out, so that meant a fourth brain surgery to remove it, then more waiting—waiting for pathology results, waiting for a second opinion, waiting for genetic analysis, waiting for the radiation plan to be developed so treatment could begin. All you want is to make everything all better for your child, and you want to do it right now. It felt like forever, but the time passed and radiation treatment began. We hoped and prayed this would be the "silver bullet" we needed to take care of this tumor once and for all. It was a long summer, but six weeks of radiation came and went, and when treatment was done it was time to wait. The plan was another MRI six weeks after radiation ended, so once again we found ourselves trying hard to put it out of our minds and get back to normal, even if just for a little while.

Since his diagnosis five years ago, our new "normal" now includes a very busy September, doing all we can to increase awareness of child-

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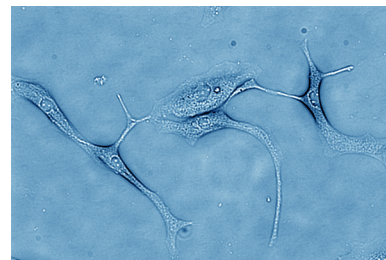
Laboratory Update

Mark Souweidane, M.D., and
Jeffrey Greenfield, M.D., Ph.D.

These are hopeful times in the CBTP laboratory—or laboratories, as we should say, since we now have four teams here. These teams work together and separately, on both individual and collaborative projects, in our quest to make new findings.

Dr. Nadia Dahmane, who joined the department this year, manages a team of researchers working on understanding how the normal nervous system and brain tumors develop. We have long suspected that pediatric brain tumors, which arise during a period of brain growth, may result from errors during this normal biological process. Therefore, understanding how the normal brain grows is a critical step for understanding the mechanisms controlling brain tumor formation, maintenance, and progression.

Dr. Dahmane's group focuses on the mechanisms controlling gene expression during the production and specification of cells in the developing brain. Gene expression is regulated by proteins called transcription factors, and part of her research projects focuses on one specific transcription factor (RP58) that her group has identified as being critical for brain growth and the generation of neurons. This protein has also been linked to brain tumor development. Dr. Dahmane's group aims at deciphering how this transcription factor works to make functional brain cells, including neurons, and the consequences of its deregulation in brain cancer cells such as those in the image at right.



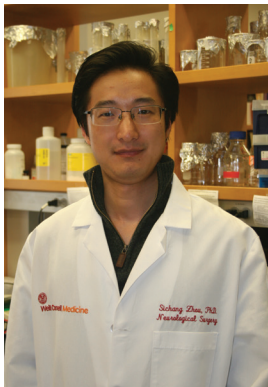
Dr. Dahmane's group is also interested in understanding how medulloblastoma, a malignant pediatric tumor arising in the cerebellum, becomes more aggressive and metastasizes to the rest of the brain. They have generated a unique and novel mouse model of medulloblastoma metastasis that will be essential in understanding the mechanisms controlling medulloblastoma progression—and, we hope, to provide novel efficient and safe approaches for therapy.

Please be sure to read our special message on page 4 of this issue. Although donations in our fiscal year (which ended in June) were up over the previous year, we see a troubling downward trend in this calendar year. We need to make up a shortfall of more than \$100,000, quickly, to get us through the end of December. We count on you all so much, and we wish we didn't have to ask you to dig deep once again. But we do. Please help us keep the momentum going by being as generous as you can.

Onward,

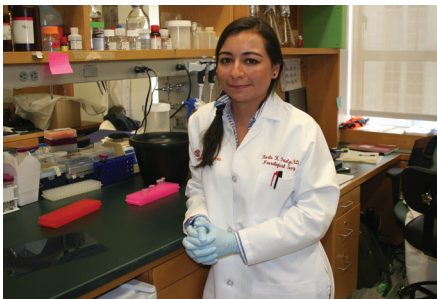
New Faces, New Projects in the Lab

The CBTP lab staff has grown dramatically over the past six months, with new research initiatives under Dr. Souweidane, Dr. Greenfield, Dr. Dahmane, and now Dr. Babacar Cisse.



Sichang Zhou, Ph.D., joined the lab as part of Dr. Babacar Cisse's team. Dr. Zhou, whose research focus is on the manipulation of stem cells, earned his Ph.D. in 2011 from Wayne State University in Detroit by defending studies of stress response of stem cells on lineage choices. Dr. Zhou previously worked at the University of Michigan in Ann Arbor to establish an in vivo mouse model of how medulloblastomas originate. Dr. Zhou is now dedicated to deciphering the molecular mechanisms involved in the transcription regulation of immune cells in brain tumors in both human samples and mouse models.

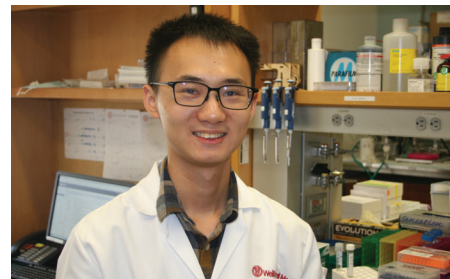
Karla Fietze, Ph.D., is a postdoctoral fellow on Dr. Nadia Dahmane's team. Dr. Fietze was awarded her Ph.D. in Molecular Biology from Princeton University in June 2017. Her dissertation was in the field of neuroimmunology, in which she studied how classical immune proteins function in the central nervous system. At the Children's Brain Tumor Project, Dr. Fietze is focused on studying the role of the transcription factor RP58, an essential regulator of brain development and brain tumor progression. In particular, her main project is using a biochemistry approach to decipher the precise molecular mechanisms involved in RP58 function both during normal brain development and in brain cancer cells.



Birra Taha is our current medical student fellow, whose work is made possible through the generosity of the Rudin Foundation. Birra is taking a year off between his third and fourth years of medical school here

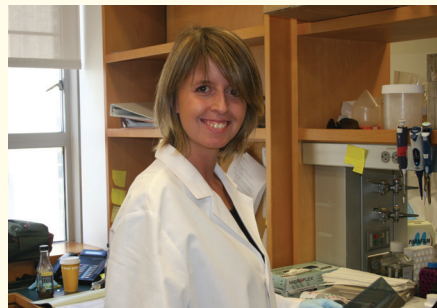
at Weill Cornell Medical College. He will be working to characterize the genomic and epigenomic dysregulations in gliomatosis cerebri and other pediatric CNS tumors (especially DIPG and ependymoma).

Meng Huang, a visiting graduate assistant who is new to Dr. Greenfield's team, is focused on the cellular and molecular mechanisms involved in the development of gliomatosis cerebri. In particular, he is working on a functional analysis of newly identified potential regulators of these tumors. Meng will investigate the function of these regulators during the developmental stages of tumor growth as well as in full-blown cancer. In addition, his project will explore the role of immune cells in the progression of low-grade glioma using primary cultures, animal models, and advanced genomics.



Meet the New Ty Louis Campbell Fellow

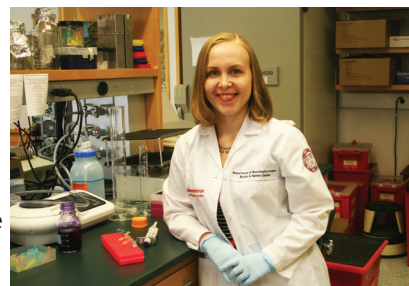
Dr. Carolina Cocito is the new Ty Louis Campbell Fellow on Dr. Greenfield's team, made possible through dedicated funding from the Ty Louis Campbell Foundation. Dr.



Cocito was awarded a Ph.D. in Neuroscience from the University of Turin in Italy, where her doctoral research focused on the role of neuronal migration and apoptosis during neurodevelopment. Her project at the Children's Brain Tumor Project focuses on the role of immune cells in glioma progression.

Testing Panobinostat for DIPG

Research technician **Julia Voronina** is leading an exciting panobinostat project at the CBTP laboratory in collaboration with a neuro-oncology group at the National Cancer Institute. In 2015, Dr. Michelle Monje at Stanford University published a study that showed panobinostat to be effective against DIPG in cell lines and animal models. The excitement over these findings was tempered by the ongoing challenge of getting even such a promising drug to the DIPG tumor site. In this study, CBTP researchers use convection-enhanced delivery (CED) to deliver the drug into the brainstem of small laboratory animals in order to evaluate the safety of this treatment, investigate its pharmacokinetic properties, and determine whether a single dose of the drug could be retained in the brain long enough to diminish the cancerous cells. Together, these research efforts will help pave the way for a future clinical trial of panobinostat using CED in children with DIPG.



A Memorable Summer

Angelia Lobifaro spent some time this summer observing Dr. Souweidane in his clinical and research work. Angelia lost a young cousin, Olivia Boccuzzi, to a brain tumor, a tragedy that has inspired her to pursue a career in medicine.

Family Update



CRISTIAN RIVERA FOUNDATION

This summer, the foundation hosted its second annual Freestyle Flashback Benefit Boat Ride to benefit the Children's Brain Tumor Project. In September, NBC anchor Darlene Rodriguez and her husband David once again offered up their home to host their annual fundraising event.



TEAM LITTLE OWL

The first-ever Hoot Under the Stars, hosted by Kyle and Kelly Fisher and Team Little Owl, raised \$45,000 for research. The event commemorated what should have been Allie Fisher's eighth birthday.



McKENNA CLAIRE FOUNDATION

The Sixth Annual Play It Forward Golf Tournament in October took place under rainy skies this year, but Macky still shone. The event was held at the Tijeras Creek Golf Club in Southern California.



ELIZABETH'S HOPE

Thank you to Claudia Amboss and Peggy Theiss for organizing the Bronxville Field Club Round Robin Event for Cancer Research to benefit Elizabeth's Hope and the Children's Brain Tumor Project. More than 100 women attended this enormously successful event, featuring tennis, bridge, lunch, a fashion show, and auction. The event was capped by a surprise appearance by Patrick McEnroe, who generously donated tennis lessons to the auction.



LYONHEARTED FOUNDATION

The Second Annual Kickball Tournament was a huge success; the Lyon family donated \$10,000 to the CBTP to fund a new study of cell-free tumor DNA in the cerebrospinal fluid (CSF) as a biomarker of disease in children with DIPG.



TY LOUIS CAMPBELL FOUNDATION

The "dirty dunk" is everyone's favorite part of the annual summer MessFest, hosted by the TLC Foundation and its Muddy Puddles Project. Then in September, the foundation hosted the fifth annual TyAthlon at Lake Mahopac, New York. Proceeds support the Ty Louis Campbell Fellowship at the CBTP (see opposite page).

SAVE THE DATE!

We are in the planning stages of a unique event to raise funds for the Children's Brain Tumor Project, so please pencil in the evening of May 1, 2018, for this very special occasion. Details will be forthcoming soon, but be sure to save the date!



REMEMBER: If you hold an event, email details and photos to info@childrensbraintumorproject.org so we can include them in our next newsletter.

Upcoming Events

- November 11, 2017: Music for Life, A Concert for a Cause; Waldorf, Maryland (Joshua Bembo Project)
- November 15, 2017: Cristian Rivera Foundation 9th Annual Celebrity Gala; Capitale (130 Bowery), New York City
- November 19, 2017: 3rd Annual McKenna Claire Foundation POKER at the BEACH; Huntington Beach, California
- November 19, 2017: Jingle & Mingle, Shop 'til You Drop Holiday Vendor Fair; Hopewell Junction, New York (TLC Foundation)
- March 10, 2018: The Kentucky Derby Gala to benefit the TLC Foundation; Old Greenwich, Connecticut

Be sure to check the CBTP calendar for details and updates. If you're planning an event, let us know—we'd love to include it!

ChildrensBrainTumorProject.org/cbtp/events/

Joe Girardi's Triple Play



No one will miss Joe Girardi quite like our families will. In three postgame press conferences this summer, Girardi spoke out about Elizabeth's Hope and the Weill Cornell Medicine Children's Brain Tumor Project, the Lily Larue Foundation, and the Ty Louis Campbell Foundation. We are grateful to him for making it a personal mission to help promote worthy causes like ours, and we wish him well.

- Joe Girardi -

The Children's Brain Tumor Project's simple goal is to give hope to hundreds of patients and families each year who experience the heartbreaking diagnosis of an inoperable brain tumor. Simply, there is not enough research funding to find a cure for this. It was inspired by a 19-year-old girl who passed away in 2012. Her name was Elizabeth Minter, and her surgeon has taken on this project. The funds are raised by families, friends, and acquaintances of these young brain tumor patients. Their whole goal is to give hope to these children, adolescents, and young adults.

We Need Your Help!

The CBTP lab has grown by leaps and bounds in the few short years since we got started, and we are so very thankful to have such a wonderful and loyal community of supporters who have made this progress possible.

It's amazing to look back to 2011, when our dream of securing a dedicated lab seemed such an immense challenge. Here we are today, with a state-of-the-art facility of our own and neuroscientific pioneers who are making progress in fighting our fight every day—all thanks to you.

As our ambitions have grown, so has our operating budget—now at \$1.4 million annually. Around a third of our funding comes from grants, but the bulk of it comes from donors like you, our most loyal supporters. You are the generous, hardworking individuals who not only dig deep themselves, but who hold bake sales and host cocktail parties, who organize and run 5Ks (and marathons!), and who ask their own friends and family to sponsor them in order to support us. We are so deeply grateful to everyone, from schoolchildren to grandparents to runners, who bring in much-needed funds to run our lab. We need you so much, and we rely on you endlessly.

We are deeply grateful whenever one of our families sends us a gift, but those donations arrive on an irregular basis, as families and friends send us the proceeds of their fundraising events. Nevertheless, our expenses are constant. Our researchers need to be paid, our supplies replenished, and our equipment purchased and maintained.

This is a particularly big challenge this year. In the first 10 months of 2016 we raised slightly over \$500,000—midway to our \$1M target. At the same point this year, we are just under \$400,000 in donations to date. This \$100,000 shortfall is significant, but not insurmountable.

Which is why we must turn to you again and ask you to help us keep going. We have some upcoming events in the pipeline, but they won't take place in time to help us meet our monthly expenses.

We would be extremely grateful for help making up the shortfall, and we ask you to be as generous as you possibly can. A secure online gift can be made at childrensbraintumorproject.org, or you may send a check to the CBTP at 525 East 68th Street, Box 99, New York NY 10065. We are so thankful to you for your support.

The Waiting

continued from page 1

hood cancer and raise money to support the research at the CBTP. It's research I hoped we would never personally need again, but it's research that we are now counting on to find a way to treat our 10-year-old son, and that we're waiting on to find a cure! These awareness and fundraising activities had a new urgency and significance to me this year, but were also a welcome distraction to help the time pass while we waited. October finally arrived and it was MRI time again, and once more we didn't get the news we had hoped to hear. There is another new spot on his brain. Like before it is small, and like before they aren't sure what it is—it might be a new tumor or just side effects of radiation. He'll have another MRI in early December, and until then all we can do is wait....



Follow the CBTP on Facebook

Brenda

Elizabeth's Hope and the Children's Brain Tumor Project

The CBTP was founded in 2011 when the dedication of Drs. Mark Souweidane and Jeffrey Greenfield, neuroscientists at Weill Cornell Medicine Pediatric Brain and Spine Center, met up with the determination of Elizabeth Minter, a remarkable young woman diagnosed with gliomatosis cerebri. Inspired by Elizabeth, Drs. Souweidane and Greenfield joined forces on a monumental task: finding new treatment options for rare and inoperable brain tumors that strike children, adolescents, and young adults. These tumors strike "only" a few hundred patients a year, so they don't get federal funding or support from larger foundations. That's why we say the CBTP is "powered by families"—because families know all too well that even one child is too many to lose.

elizabeth's hope



Elizabeth Minter (1991-2012)