



Children's Brain
Tumor Project
powered by families

May 2014



Elizabeth Minter (1991-2012), Founder of Elizabeth's Hope

From the Desk of...
Denise Downing

Last summer, my 10-year-old daughter Courtney and I made our way from Orlando to New York for a Children's Brain Tumor Project event. We decided to arrive a day early in hopes of getting to see Train at the Today show; they were performing as a part of the summer concert series. Lucky us—a friend got us VIP tickets to the show!



Denise Downing and her daughter Caitlin, the first patient treated in Dr. Souweidane's clinical trial for DIPG.

Courtney was determined to take advantage of this opportunity to tell everyone she could about our purpose in New York and the commitment our family had made to an important cause: the Children's Brain Tumor Project. She became engaged on a mission to take a poster about her sister Caitlin to the concert.

Roseann Henry, friend to many who are familiar with the Weill Cornell Pediatric Brain and Spine Center, offered to assist Courtney in making the poster and getting it printed fast so we could take it to the concert less than 24 hours later.

As Courtney sat next to Roseann, busily creating and typing at the computer, I stood tucked in the corner of the tiny office and, in a rare moment when I thought of nothing, I watched the poster process unfold in front of me. They picked fonts and sizes. They chose words with enthusiasm. They looked at different colors and shapes. They arranged and re-arranged. And then they chose the logos to go on the poster.

"Cheering for Caitlin" was first, with bright pink letters in a girly font with a rainbow as a backdrop. Next, the Children's Brain Tumor Project logo: Two orange figures holding hands with a smaller blue one below a bright yellow sun, the whole thing heart-shaped to represent families cradling their little ones with love. Next to that in blue print were the words "Children's Brain Tumor Project" and beneath that in slightly smaller print it read "powered by families."

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Laboratory Update

Mark M. Souweidane, M.D.
Co-Director, Children's Brain Tumor Project

I'll never forget May 1, 2012—that was the day I performed the very first procedure in a new clinical trial testing convection-enhanced delivery (CED) of a therapeutic agent in a child with DIPG. More than ten years of my prior lab work, including bench research and animal testing, had me absolutely convinced that this was a safe procedure. Still, with anything so completely new there's just that tiny seed of doubt, the faintest whisper of "what if..."



What I was not prepared for were the emotional ties that quickly took hold of me. The little girl in the OR that day was Caitlin Downing, whom I had come to love in just a few short weeks of knowing her and her family. Hugs, insightful questions, and smiles from this 5-year-old were gripping. Caitlin's sister, Courtney, had even joined her on one of her preoperative visits to New York so that she could approve of the doctor who would offer hope.

Imagine my relief when the procedure went exactly as expected, and imagine my heartbreak several months later when the tumor came back to claim Caitlin's life. But her successful surgery validated every effort that went into this translational project and left no doubt that we should push on.

Today we find ourselves in an exciting position: With 13 children treated so far (all of whom have tolerated the treatment well, with no adverse effects), we have answered critical questions about the technical, surgical, and diagnostic aspect of this drug delivery tactic. Early results have raised awareness from oncologists, neurosurgeons, radiologists, and pharmacologists from all over the world. The momentum is building rapidly.

So encouraging are the results that we have decided to request a continuation of the study. If the continuation is approved, we will plan to treat a minimum of 12 more

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Laboratory Update

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children at escalating doses. The study is being positively viewed on a national level. The preliminary results of this trial were presented to the Pediatric Brain Tumor Consortium (PBTC) in Atlanta last month. That group is highly interested in moving the strategy into a "group-wide" study and has assembled a working group, which I am leading, to formulate the best plan.

Even as we modify the current strategy, we are continuing our laboratory and clinical efforts in different arenas. As an example, we are using other strategies for bypassing the blood-brain barrier, which is what prevents us from getting adequate doses of cancer-fighting molecules to a tumor site. In addition to testing CED, we are also pursuing another potential route, using super-selective intra-arterial chemotherapy to deliver medicine directly into the blood vessels of the tumor itself. That new clinical trial is actively recruiting patients, with the first patient treated last month. We will explore every avenue to work toward the same ultimate goal.

We also know that existing worldwide talent offers us a huge potential network that might contribute to therapeutic discoveries. The collaborative clinical effort through Memorial Sloan-Kettering Cancer Center has allowed us to bring this concept to reality at an institution that is no stranger to innovative cancer therapy. Using the best available animals models for DIPG has led us to collaborate with Dr. Oren Becher at Duke University. In fact, Ranjodh Singh, a Cornell medical student, will spend the coming year to use Dr. Becher's models to test combinations of small molecule inhibitors (drugs that are specific for the molecular driving force of DIPG).

The most recent work of the DIPG Collaborative has led toward DIPG-specific drugs that interfere with the most common genetic and epigenetic changes in these tumors. Dr. Michelle Monje at Stanford University, a renowned expert in defining the origin of DIPG, is working with us to validate some of her most recent laboratory work showing highly promising tumor response rates. We have also recently been connected with experts in nuclear medicine imaging at Yale University who would like to explore the option of using brain-specific EPT imaging on patients treated in our clinical trial continuation. This exciting work represents the most sensitive methods available for assessing drug concentrations in the brain stem of children after treatment with CED.

We are light-years beyond where we were just a few short years ago when I first entertained an idea that many said was fraught with theoretical dangers, technical impossibilities, and prohibitive expenses. We are now at a crossroads and will take the path that merges our experience with worldwide expertise. We will continue to use every avenue possible to avoid another child asking if "Dr. Mark can remove the bump in her brain before she goes to heaven," as Caitlin did after her groundbreaking surgery.

Mark M. Souweidane, M.D.

Reinforcements Are on the Way!

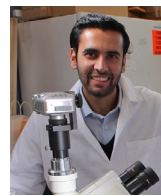
We're delighted to introduce some new faces here at the CBTP, joining the team thanks to the generosity of our donors. As we proved last year, when Emma Vartanian's summer fellowship helped lay the groundwork for a \$400,000 Department of Defense grant, these additional resources can pay off many times over:



Allison Settler, MSN, FNP-BC, is our new liaison for pediatric brain tumor clinical research, charged with connecting patients with clinical research programs. Having Allison here will simplify the process for families and improve patient enrollment.



Babacar Cicce, MD, PhD, is a neurosurgical resident who will spend the next 18 months in Dr. Greenfield's lab working on interactions between tumor microenvironment and the immune system, which was his PhD topic.



Ranjodh Singh, who already volunteers in the CBTP lab in his "spare" time, will take a year off from medical school to work in Dr. Souweidane's lab full time. Ranjodh will return to school in September 2015 to complete his medical degree.



Scott Connors is taking a year off between his third and fourth years of medical school to work in Dr. Greenfield's laboratory, using precision medicine techniques to study tumor progression after multiple resections.

William Cope will spend the summer between his second and third years of med school exploring how low-grade glial tumors transform into high-grade ones.

Beiye Shen, PhD, will spend the summer researching intercellular communication and signaling between tumor cells and blood vessels at the blood-brain barrier.

The Ty Louis Campbell Fellowship

The Ty Louis Campbell Foundation has generously pledged a two-year, \$130,000 grant to fund a new research fellowship for the CBTP. The Ty Louis Campbell Fellow will support Dr. Greenfield's research in genomic sequencing and precision medicine. In announcing the fellowship, Lou and Cindy Campbell, Ty's parents, said, "We feel that Dr. Greenfield's pioneering research deserves our support to ensure that the momentum he has spearheaded to find a cure continues." The Ty Louis Campbell Fellow is expected to join the research team effective July 1, 2014.



Event Highlights

Annie Longobardo and her mom, Lisa, dedicated their run in the Michelob Ultra New York 13.1 Marathon on March 22 to **Elizabeth's Hope** and raised more than \$2,000 for the Children's Brain Tumor Project. Thanks, Annie and Lisa, for yet another great effort on our behalf!



Katie Chandler was named Miss Winter Park's Outstanding Teen in January, with the theme "Finding a Cure for DIPG" in honor of **Caitlin Downing**. Katie advances to the Miss Florida's Outstanding Teen Pageant in June, when she will speak about the Children's Brain Tumor Project. Katie will be accompanied by a "little princess," played by Caitlin's big sister, Courtney. Both pageants are part of the Miss America Scholarship pageants.



The good folks of Oviedo, Florida, came together for a fund-raising Rummage Sale in memory of **Caitlin Downing...** six-year-old twins Eve and Danya Herman of New York asked for donations to the Children's Brain Tumor Project in lieu of birthday presents, in memory of their friend **Fiona Lundell...** friends and colleagues of Tom Jones, who dedicated his August 2013 Mt. Kilimanjaro climb to **Elizabeth Minter**, have continued to spread the word and show their support, with thousands of dollars in additional donations arriving this winter... Gretchen Scott, who has pledged all of her proceeds from the **Elizabeth's Hope** shirt to supporting our research, sent in another generous donation from these sales... the **McKenna Claire Foundation** made a \$50,000 gift, proceeds of their many fund-raising events, to support the Children's Brain Tumor Project's research on DIPG, the tumor that claimed their daughter's life... Huge thank yous to everyone who supports the Children's Brain Tumor Project!

Coming Up

See childrensbraintumorproject.org for details about all events.

- May 2: Party With a Purpose (Huntington Beach, CA)
- May 16: Rocking for Research (Dallas)
- May 17: Christian Koehler Lacrosse Tournament (Islip, NY)
- May 18: Caitlin Downing Remembrance 5K Run/Walk (Oviedo, FL, and nationwide virtual walks—sign up at cheeringforcaitlin.com)
- May 18: BANDs Together Against Brain Tumors (Randolph, NJ)



SAVE THE DATE: Mark your calendars for the first annual CBTP 5K/10K and Family Festival on Saturday, **September 13, 2014**, in Bronxville, New York.

If you hold an event, be sure to email details and photos to info@childrensbraintumorproject.org so we can include you in the next newsletter!

You Did It!



In our December 2013 newsletter we told you how close Elizabeth's Hope had come to raising its first \$1 million, and we urged our supporters to help us reach that milestone. We're both proud and humbled to say that we made it, thanks to you. We are dedicated to continuing our work supporting the Children's Brain Tumor Project, and we are so thankful to know that you are behind us every step of the way. Thank you!

Mike *Emmie*

Powered by Families

A special kind of magic happens when the families we're working with meet one another. We'll never forget Brenda Ries and all of TEAM Sean showing up at a race last year wearing rainbow socks in honor of Caitlin Downing. Or Enza and Frank Boccuzzi at the podium at the Cristian Rivera Foundation's annual gala talking about their daughter, Olivia. Or Kathleen Clark coming all the way from Louisiana to meet "Miss Emmie" Minter—bringing with her a check for the proceeds of her fund-raising in memory of her father.

These moments were powerful indeed. What if, we wondered, we brought all of "our" families together for a day? What if everyone could meet and share stories, learn more about our research projects and clinical trials, and brainstorm new ideas?

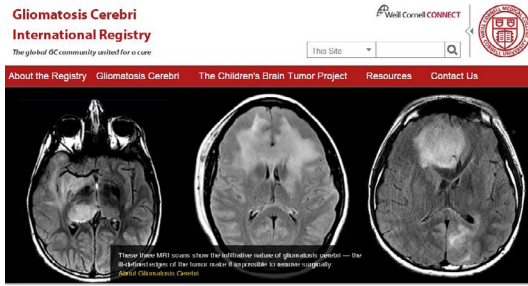


That's what happened on March 19: Twenty-eight family members came to New York for an inspiring, informative, and highly emotional summit. We heard from Neurosurgeon-in-Chief Dr. Philip Stieg on the dismal state of federal funding for pediatric brain tumors. Drs. Mark Souweidane and Jeffrey Greenfield presented the latest updates on their laboratory research and clinical trials. Families reported on their social media outreach, fund-raising efforts, and awareness events. And everyone got to meet Mike and Emmie Minter, whose amazing daughter, Elizabeth, was the original spark for a project that has turned into a powerhouse.

Over the coming months we'll be working with our Family Council on new projects, new awareness events, and new fund-raising efforts. We know there's one thing they'll do best—and that's to remind us, every day, about the very real human cost of these rare and inoperable tumors we're fighting. Nobody knows better than they do how important this work really is.

A Sneak Preview of the GC International Registry

Thanks to the dedication of Joshua Bembo's family and the generosity of The Joshua Project, we recently launched an important new Web site at GCRegistry.com. The site allows patients to sign up for an international gliomatosis cerebri database, which will for the first time give researchers all over the world access to information and tumor samples from the cancer that claimed Joshua's life. We will be publicly announcing the new registry soon, but you can take a sneak peek at it now. Look for more information in our next issue.



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



The CBTP was founded in 2011 when the dedication of Dr. Mark Souweidane and Dr. Jeffrey Greenfield, neuroscientists at Weill Cornell 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.

Ways to Give

1. By Check

You may make your gift by check, payable to Weill Cornell Medical College. Please indicate "Children's Brain Tumor Project" in the memo area.

Please mail checks to:
Ana Ignat, Dept. Administrator
Weill Cornell Pediatric Brain & Spine Center
525 East 68th Street, Box 99
New York, NY 10065

2. By Credit Card

Visit childrensbraintumorproject.org to use a credit card to make a secure online donation or a monthly pledge.

3. Stock, Matching Gifts, and Other Options

To discuss corporate matching programs, donations of securities, or other options, please contact Ana Ignat, Department Administrator, at ani7003@med.cornell.edu.



Follow the CBTP on Facebook

From Denise Downing

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That's all it said. As they were finishing it up they turned and asked my opinion. My mouth dropped in astonishment, thinking they had forgotten something. "What about the rest of the logo?" I asked.

I explained that my undergrad degree in psychology and graduate degree in counseling, coupled with years of working in a teaching hospital, had taught me that when you're promoting any kind of research, the name of the individual or organization responsible is always prominently displayed. But I didn't see Weill Cornell's name. I didn't see Dr. Souweidane's name or Dr. Greenfield's name.



Roseann explained what I had already sensed and had long felt in my heart: Dr. Mark Souweidane and Dr. Jeffrey Greenfield don't care about their names being blazed on posters and banners for all to know. They aren't after notoriety or fame. They just want to treat children with brain tumors. They just want to make a difference in the fight that too many families find themselves battling each year when they are shocked by a brain tumor diagnosis.

They just want kids to stand a fighting chance.

Thank you, Dr. Souweidane and Dr. Greenfield, for your selfless commitment in fighting to find a cure for our children.

May Is Brain Tumor Awareness Month!

Chances are that if you're reading this newsletter you already know of a family affected by a child's brain tumor. Did you know how terribly meager the funds are to research these tumors in kids? This May, consider attending an event, making a donation, and—most importantly—speaking up to let everyone know how important it is to support this cause! You can carry our logo with you to spread the word by shopping at our online store.



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