



# Children's Brain Tumor Project

powered by families



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

August 2014

From the Desk of...  
*Cindy Campbell*

My husband and I were introduced to the world of pediatric brain tumors when our perfect, beautiful little boy, Ty, was diagnosed with a rhabdoid tumor on the brainstem at 2 years and 10 months old. He died 13 days after his fifth birthday in October 2012 and we are left to pick up the pieces of our broken lives.

Throughout Ty's journey, and ever since his loss, Lou and I have been actively involved in getting to know other families like ours, and getting to know the doctors who are committed to helping these children through much needed, grossly underfunded research and clinical trials. We are in constant awe of the men and women who have devoted their lives to pediatric neuro-oncology and pediatric neurosurgery. Doctors like Jeff Greenfield and Mark Souweidane.

Of course, there are thousands of scholars, researchers, and doctors who selflessly devote their time to helping others. Searching for a cure. But let me tell you why a career in pediatric neuro-oncology is so very remarkable to us both.



First and most obviously, because we are talking about children. Little babies who don't understand why magic kisses can't make their boo-boos go away. Many of these veterans (including Jeff and Mark) have small children of their own, and instead of being turned off

by how difficult it is to imagine their own children as patients, they are inspired to make sure these children are getting the best possible care and hope for a future. It takes a very special heart to be so devoted to a cause that may frighten others.

Then there is the reality of pediatric brain tumors. When a brain or spinal tumor is involved, the detrimental effects are that much more severe. The sad reality of pediatric brain tumors is that they steal so much from the child. The ability to speak, eat, walk, play, and more can all be seen slowly slipping away as tumors progress. Brain and spinal cord tumors are the second most common of all cancers among children, and brain tumors include the deadliest of all types of childhood cancers (with survival rates of 0% in many cases).

When a child is diagnosed with a brain tumor, the deterioration

(continued on page 4)

## Laboratory Update

Prajwal Rajappa, M.D.

Pediatric Brain Tumor Fellow

*Weill Cornell is one of 200 institutions that make up the Children's Oncology Group (COG), the world's premier organization dedicated to eradicating childhood cancer. Dr. Mark Souweidane and Dr. Prajwal Rajappa lead the Weill Cornell Pediatric Brain and Spine Center's participation in a long-term study of brain tumor tissue samples collected from pediatric patients around the world, with the goal of understanding the molecular profile of these tumors. This lab update about our COG project was contributed by Dr. Rajappa.*



This COG study is extremely important given the lack of effective treatments available for pediatric brain tumor patients. We are collecting brain and spinal tumor tissue from pediatric patients treated at COG facilities around the world, providing a repository for long-term storage of those specimens. This study allows us to make specimens available to expert researchers both here in the United States and abroad who are working to understand the biology of these tumors.

These repository-driven studies utilizing human tissue serve as the platform for meaningful translational research, which relies on laboratory findings that can be quickly applied to develop new treatment options. The rapidly evolving science of human genomics has the potential to change how physicians diagnose and treat cancer, but molecular studies require access to tumor tissue, which has been extremely limited—especially when the tumors are rare. There's a particular shortage of pediatric tissue specimens available for ependymomas, thalamic gliomas, brainstem gliomas, diffuse pontine gliomas, gliomatosis cerebri, low-grade gliomas, and primary GBMs. The limited number of specimens available for research within any single institution can make it impossible to conduct statistically powerful and high-impact molecular research. Therefore, participation from multiple institutions is essential.

Here at Weill Cornell, with a world-class pediatric service that draws patients from near and far, we have gathered a wide variety of samples of many pediatric brain and spine diseases in our repository, making us one of the top member institutions in the COG. In fact, in our first full year of participation in this study (2012), Weill Cornell ranked eighth

(continued on page 2)

**Make a donation today at [ChildrensBrainTumorProject.org](http://ChildrensBrainTumorProject.org)**

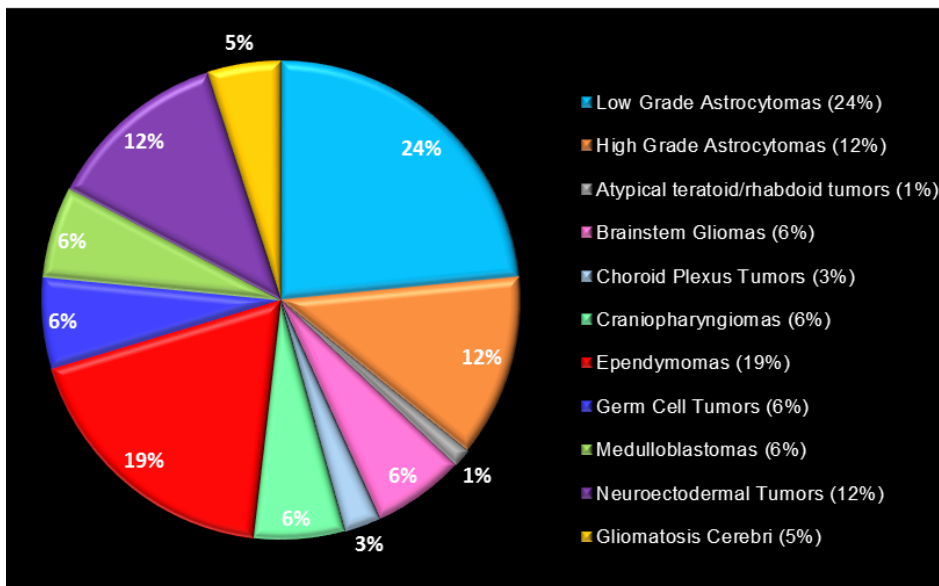
## Laboratory Update *(continued from page 1)*

out of 142 participating institutions in terms of number of pediatric patient samples enrolled. This year, we are poised for our highest patient enrollment on study. As shown in the pie chart at right, we have accrued a wide array of pediatric central nervous system (CNS) disease entities in our departmental repository that will serve as valuable material for further genomic sequencing and translational research.

Within Weill Cornell, we have developed streamlined collaborations with other departments, such as the department of pathology, which will ultimately advance our understanding of the biology of pediatric brain tumors and further initiate multi-disciplinary studies. Solidifying our role at COG, at Weill Cornell, and within the cooperative group setting may also serve as a mechanism for securing federal funding sources.

Today, encouraged by our success with both the national protocol and our multi-disciplinary collaborations in place here at Weill Cornell, we embark on a regional effort that will bring institutions from the tri-state area together to facilitate scientific progress. To provide our patients with the very best therapeutic regimens, it is paramount that we consolidate our tissue resources in a multi-institutional pediatric brain and spine tumor repository in the New York area. We have proposed the creation of a scientific advisory panel to regulate the flow of tissue for various scientific proposals that will be evaluated for investigator scientific merit, publication profile, and preliminary data. This proposal is currently under review by the Weill Cornell Medical College Institutional Review Board (IRB) and we aim to start accruing patient specimens regionally by early this fall. Having adequate samples available will facilitate work to be done by Dr. Jeffrey Greenfield and Weill Cornell's Institute for Precision Medicine in Pediatrics. In an exciting new study approved just this month by the IRB, Dr. Greenfield and the Precision Medicine team will seek to develop tumor-specific targeted therapies.

Our participation in the pediatric neuro-oncology cooperative group, along with enhancing our own intra-institutional collaborations, is of utmost importance as we seek new treatments for currently incurable pediatric brain tumors. This collaborative approach to pediatric brain tumors is critical to helping us find answers that will provide hope to these children and their families. We at the Weill Cornell Pediatric Brain and Spine Center express our sincere gratitude to the families in sharing a vision for a future where effective treatment options can be afforded to these children. With your support of our research initiatives, we actively work towards that brighter day.



**Spectrum of brain and spinal tumors that comprise the Weill Cornell Pediatric Central Nervous System Tumor Repository, as of Summer 2014.**

## DIPG Clinical Trial Continuation Wins FDA Approval



The Food and Drug Administration (FDA) has approved a continuation of Dr. Mark Souweidane's Phase 1 clinical trial for diffuse intrinsic pontine glioma (DIPG). The original clinical trial, which began in May 2012 with Caitlin Downing, has treated 14 patients using convection-enhanced delivery (CED) of a therapeutic agent in increasing dose levels; the approved continuation adds three additional dose levels. The approval re-opens the trial for new patient enrollment.

The clinical trial, which was developed in Dr. Souweidane's lab at Weill Cornell and is being conducted at Memorial Sloan-Kettering Cancer Center (MSKCC), takes a new approach to delivering cancer-fighting drugs to a pediatric brain tumor. Chemotherapy historically has limited effectiveness on brain tumors since drugs delivered to the bloodstream (either in pill form or via IV drip) cannot cross the protective blood-brain barrier. In Dr. Souweidane's trial, a therapeutic agent is delivered directly to the tumor by way of a tiny catheter.

More details about the trial are available at [childrensbraintumorproject.org](http://childrensbraintumorproject.org).

## GC Registry Is Live

The Gliomatosis Cerebri International Registry web site, donated by the family of Joshua Bembo, is now live at [GCRegistry.com](http://GCRegistry.com). A launch announcement was mailed to 20,000 neurosurgeons, oncologists, radiologists, and other specialists around the United States to ensure widespread awareness of the registry. Dr. Greenfield's team is now accepting and processing requests for registry participation from patients, family members, and health care providers. The CBTP is deeply grateful to The Joshua Project for making the web site possible.



# Event Highlights



**Elizabeth's Hope** celebrated spring with a fundraising cocktail party on April 25 at the stylish Bowery Hotel in New York.

All 600 tickets sold out well in advance, and the event raised more than \$40,000 for the Children's Brain Tumor Project. Many thanks to Tessa Naso for organizing another winning event.



Our CBTP families were busy in May for Brain Cancer Awareness Month. On May 4, the **Joshua Bembo Project** walked in the Race for Hope in Washington, D.C. (left) On May 17, the **Christian Koehler Foundation** held its annual lacrosse tournament in East Islip, Long Island, donating this year's proceeds to the CBTP.

**TEAM Sean** held its annual BANDs Together Against Brain Tumors event on May 18.

Also on May 18, **Cheering for Caitlin** put on a 5K Remembrance Walk in honor of what should have been Caitlin's 7th birthday.



The event was hosted in Caitlin's home town of Oviedo, Florida (left), but walkers there were joined by "virtual" participants from all across the country. (Inset: participants in Pennsylvania.) More than 1,000 people walked in this event nationally, raising more than \$20,000 for the CBTP. Many thanks to Katie Chandler and her mom, Joni Campbell Chandler, for organizing this event. Katie even went on to bring her DIPG awareness campaign to the final round of the Miss Outstanding Teen Florida pageant.



On June 7, **Olivia's Foundation** hosted the Curing Kids' Cancer Marathon in Brooklyn, where Olivia's parents, Enza and Frank Boccuzzi, presented Dr. Souweidane with \$50,000 to support his research. Our deepest thanks for this tremendous generosity.



**Christopher Collina's** parents marked the May 21 anniversary of his diagnosis by holding t-shirt sales at PS158 in Staten Island (where his mom Melissa works) and the Police Department in Plainfield, New Jersey, (where his dad Tommy works). The Collinas sold hundreds of shirts and donated the proceeds to the Children's Brain Tumor Project.

## Coming Up

See [childrensbraintumorproject.org](http://childrensbraintumorproject.org) for details about all events.

**August 9:** Muddy Puddles Mess Fest (Mahopac, NY)

**August 16:** Team Little Owl booth at Queen's Price Chopper Block Party (Overland Park, KS)

**August 23:** Architecture tour (Petra Island, Lake Mahopac, NY)

**September 13:** The Bronxville Road Race for the Children's Brain Tumor Project (Bronxville, NY)

**September** (date to be announced): Sixth Annual Cristian Rivera Foundation Celebrity Gala

**September 20:** Long Beach Luau (Long Beach, NY)

**September 27:** Second Annual TyAthlon (Mahopac, NY)

**Oct 16-18:** Second Annual Allie's Sale (Overland Park, KS)

If you hold an event, be sure to email details and photos to [info@childrensbraintumorproject.org](mailto:info@childrensbraintumorproject.org) so we can include you in our next newsletter!

## The Bronxville Road Race for the CBTP September 13, 2014



For our very first official CBTP fundraiser, we're planning a festive morning with three events:

**A 5-mile race**, the main event; medals will be awarded for first through third places in age groups from under 19 to 70+.

**A short course, of just over two miles**, is a walk/run for families and friends. Medals will be awarded for the first three participants to cross the finish line walking a dog, and the first three pushing a baby stroller!

**A Kids' Dash** of one mile, with finisher medals for all and winner medals in three age groups, offers friendly competition for kids under 12. Parents can run free with small children. The Kids' Dash will be led by Liam Greenfield, who ran last year's 5K with his dad, and William and Miles Souweidane, who ran their first races in June with Olivia's Foundation.

**Register today to run, or sponsor one of our runners, at** [childrensbraintumorproject.org](http://childrensbraintumorproject.org)

[ChildrensBrainTumorProject.org](http://ChildrensBrainTumorProject.org)



Dr. Greenfield and Kathleen Clark

## A CBTP Daughter Turned Fundraiser Turned Intern

If you had told me at the start of my freshman year in college that I would soon be interning at a major medical research center—in New York, of all places, more than a thousand miles from home in Louisiana—I would have told you you were crazy. But here I am three years later, sitting at a desk at Weill Cornell Medical College, working for the Children's Brain Tumor Project.

It's funny how one moment can change your life forever. For me, that moment came when my 50-year-old father was diagnosed with gliomatosis cerebri. My family was shocked and devastated. I researched the condition online and found *Elizabeth's Hope*, which is how I met Mrs. Emmie Minter. I reached out to her, and our friendship grew from there. Even though I was far away in Louisiana, she helped me become involved in the Children's Brain Tumor Project. She introduced me to Dr. Greenfield, and I knew that this was the right organization for me. I've never met a doctor so caring, and I felt confident that his research could make a difference. That is one of the many reasons why my family decided to donate my father's brain for Dr. Greenfield's research. Mrs. Minter also helped me get to know everyone at the Children's Brain Tumor Project, which is how I ended up here today.

This summer will be full of new and exciting things, as I help prepare for The Bronxville Road Race, the first CBTP event in Bronxville, which was Elizabeth Minter's home town. I'm hoping that I will be able to help continue to spread the word about the Gliomatosis Cerebri International Registry ([gcregistry.com](http://gcregistry.com)), because that is very important to the research. The skills and information I learn this summer will also help prepare me for my fundraising in Baton Rouge as I approach my senior year.

I am very grateful to be able to work with such an incredible effort, which truly is "powered by families." It not only raises money for rare and inoperable brain tumors, but it brings together families who have gone through such a devastating tragedy. As my dad said, "Nothing is more powerful than a family."

— Kathleen Clark

### 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](http://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](mailto:ani7003@med.cornell.edu).



Follow the CBTP on Facebook

## From Cindy Campbell

(continued from page 1)

is like nothing you could imagine or prepare for. My son, for example, was 100% healthy before the dreaded diagnosis. He met all of his developmental milestones and at 2½ years old was enjoying the use of his improving vocabulary. His sense of humor was evolving and knock-knock jokes were becoming a new favorite (although I don't think he truly understood the punchlines—at least not as much as his larger-than-life laughter would express). He loved discovering new foods to snack on and the playground was a daily triumph where he overcame fears and learned new tricks. The "high slide" was his latest feat and his adorable smile was bursting with pride as he climbed that ladder over and over again.



His cancer story starts like everyone else's. At night, he seemed uncomfortable. He didn't sleep well. Finally, it was time to do more than talk to the pediatrician, so we insisted on an MRI. Thus, it began. Slowly, over the course of two and a half years in treatment, we watched as he was robbed of all the childhood joys. From the time of his very first surgery he never walked independently again; he spent more than 250 nights in the hospital; his speech became compromised; he was often unable to swallow or clear his own throat; he was in pain more often than not; and he never had the chance to enjoy another day on the playground.

As his parents, we needed to do more than just watch. We searched high and low for new treatment options, we educated ourselves on the research landscape, and we exhausted all resources. We could not save our son, nor could his doctors... but we do believe that someday there will be another boy just like him who enjoys a beautiful future thanks to the dedicated work of the doctors and researchers at The Children's Brain Tumor Project. For that tremendous gift of hope, we are forever grateful.