



Children's Brain Tumor Project

powered by families

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Issue #1

Research News

Mark M. Souweidane, M.D.
Jeffrey Greenfield, M.D., Ph.D.
Co-Directors, Children's Brain Tumor Project

Welcome to the first issue of the Children's Brain Tumor Project newsletter, created to keep our supporters informed about the research they are funding. We are so grateful for your gifts, which make our work possible. This truly is a dream come true for both of us, and some day we hope to be able to have a significant impact on the care of children facing incurable brain tumors.

As we close out 2013, we can't help but reflect on how far we've come in just two years. Thanks to you, we have two clinical trials now enrolling patients, and we are recruiting for a new post-doc researcher and a bioinformatics fellow.



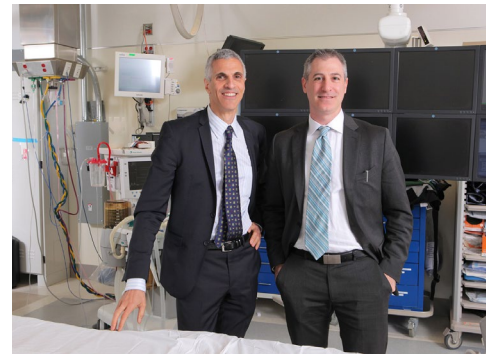
We have now treated nine young patients in the clinical trial for diffuse intrinsic pontine glioma (DIPG). That trial is testing the safety of interstitial infusion (also known as convection-enhanced delivery, or CED) of therapeutic agents for DIPG. It's still early, and we have a lot of data to analyze, but we are greatly encouraged by what we've seen so far in this trial: There have been zero instances of adverse effects to date.

Alternative delivery methods like CED are only half our two-pronged strategy at the Children's Brain Tumor Project. We believe that precision medicine, which uses the new science of genomic sequencing to "fingerprint" a tumor to determine its molecular characteristics, is key to finding the right drugs to fight it.

Best of all, we've recently been approved for another new clinical trial, this one testing superselective intra-arterial chemotherapy (SIAC) for pediatric brain tumors. This is a great milestone, as SIAC allows us to deliver chemotherapy drugs directly to the small arteries that feed a tumor, instead of into the whole body, as is the case with IV or oral chemo.

Imagine a new age of chemotherapy, in which the side effects of traditional chemo are a thing of the past. Precision medicine will someday allow us to select drugs specifically for an individual's tumor, and new delivery methods will get those drugs directly to the tumor, without toxic side effects.

We are grateful for your continued support, and we look forward to making great progress in the coming months and years.



MILESTONES

2011

Dr. Mark Souweidane applies for FDA approval for a clinical trial to test the safety of convection-enhanced delivery (CED) for DIPG. Supported by more than a decade of lab research, the application is approved.

2012

In May, Dr. Souweidane treats the first patient in his clinical trial. Testing different agents, at different doses, will generate the data we need to find the right combination.

Dr. Jeffrey Greenfield sequences the first sample of gliomatosis cerebri, which yields a treasure trove of information about the genetic mutations associated with it.

In the fall, our work converges with that of C. David Allis, Ph.D., at Rockefeller University, who is investigating a DNA histone called H3.3. When H3.3 mutations are found in pediatric gliomas, we begin collaborating on a project to find out why the mutation causes gliomas—including gliomatosis cerebri—to develop.

2013

In January, the Institute for Precision Medicine opens at NewYork-Presbyterian/Weill Cornell, bringing the ability to sequence tumors onto our campus.

The St. Baldrick's Foundation provides funding for a summer fellowship for a medical student. Emma Vartanian is able to do the groundwork for an important new project we're been planning to initiate. Which led to...

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Make a donation today at childrensbraintumorproject.org

Powered by Families

Some of the families now powering the Children's Brain Tumor Project were supporting Dr. Souweidane or Dr. Greenfield before there even was an official project; others heard about us on the Web, on Facebook, or through word of mouth. We are energized by your support, even as we mourn the loss of so many beautiful children. This is just a sampling of our families and of the events, large and small, that you have so generously organized to raise funds for our work. We can't thank you enough for your continued support.



Cristian Rivera Foundation

When 6-year-old Cristian lost his battle with DIPG in 2009, his parents dedicated themselves to funding Dr. Souweidane's research. Through an annual celebrity gala and other events, the Cristian Rivera Foundation has contributed nearly a quarter-million dollars to the CBTP.

Elizabeth's Hope

Elizabeth was 21 when she succumbed to gliomatosis cerebri in 2012. Before her death, Liz collaborated with her family and Dr. Greenfield to create *Elizabeth's Hope*, which raises funds to support the CBTP. Seeded by an extremely generous donation from Elizabeth's parents, *Elizabeth's Hope* has raised more than \$900,000 from individual donors, high school and campus events, even a Mt. Kilimanjaro climb.

Ty Louis Campbell Foundation

Ty was diagnosed with AT/RT just before his third birthday, and he passed away in 2012 a few days after turning five. Ty's parents were tireless in their search for hope for Ty, and they have been equally dedicated to funding research. The Campbells have raised tens of thousands of dollars through their Muddy Puddles Project, TyAthlon, and other fundraisers, and recently pledged to fund a two-year research fellowship.

Cheering for Caitlin

Five-year-old Caitlin Downing was the first patient treated in Dr. Souweidane's DIPG clinical trial in 2012. Caitlin and her family showed remarkable bravery in agreeing to take this pioneering step, which provided the first evidence that CED is safe. Caitlin passed away in November 2012 when her tumor returned; her family and friends have become loyal supporters of the CBTP.

Love for Buggie

Fiona Lundell, known to those who loved her as Buggie, was five when she was diagnosed with an anaplastic thalamic astrocytoma, an inoperable glioma. Her grandfather, Peter Godfrey, stepped up to lead fundraising efforts to support the Children's Brain Tumor Project and has made a very generous personal pledge to the project as well.

TEAM Sean

Sean Ries is a rare and happy success story, as Dr. Greenfield was able to remove 90 percent of his brain tumor after many other surgeons said surgery was impossible. Sean's mom has become an avid supporter of the research, organizing and participating in awareness events and fundraisers.

Families around the world have joined the effort in the name of other young patients, including **Jake Grecco**, **Juliana Donnelly**, **Christopher Collina**, **Joshua Bembo**, and **Allie Fisher**. **Kathleen Clark** is a champion for our research in her hometown in Louisiana, where she leads the efforts of "What's YOUR Fight?" on behalf of her father, Daryn, who was a rare adult victim of gliomatosis cerebri. The parents of **Lyla Nsouli** started the **Lyla Nsouli Foundation** in the U.K.

Perhaps our happiest contribution came from a previous patient of Dr. Souweidane's, who sent a gift celebrating his wedding day. With your help, we're confident we will enable many more patients to grow up to mark milestones like these!

MILESTONES (continued from page 1)

...In September, Yujie Huang, Ph.D., receives a two-year, \$400,000 grant from the Department of Defense to study how and why low-grade gliomas develop into high-grade tumors like gliomatosis cerebri. We theorize that there are signals coming from the bone marrow that cause the rapid development of the tumor's blood supply. If we can stop or slow that process, we just may be able to stop the progression to fatal tumors.

In November, we receive the green light to initiate a clinical trial using superselective intra-arterial chemotherapy (SIAC) for brain tumors in children and adolescents.

2014 and Beyond

Today we are recruiting new fellows to start as soon as this January, and we are soon to announce the launch of the International Gliomatosis Cerebri Registry at GRegistry.com. The registry will enroll patients from around the world in a comprehensive database tracking tumors, mutations, diagnoses, and outcomes. If information is power, this new registry may turn out to be one of our most powerful weapons yet in this battle.

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You may make your gift by check, payable to Weill Cornell Medical College. Please put "CBTP" in the memo area.

Please mail checks to:

Ana Ignat
Weill Cornell Pediatric Brain & Spine Center
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New York, NY 10065

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

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