



American
Brain Tumor
Association®
Providing and pursuing answers®

Headlines

FALL/WINTER 2014 | VOLUME 41, NUMBER 4

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THE ROAD TO PERSONALIZED MEDICINE *Where will it Lead Brain Tumor Treatment?*

Personalized medicine, sometimes called precision medicine, is a developing area that utilizes an individual's genetic information to help inform diagnosis and treatment decisions.

"Brain tumors arise because there are a lot of genetic changes that occur that cause cells to divide and grow."

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Message from the President and CEO

Advances in our understanding of brain tumors and a tumor's response to available and emerging therapies are beginning to expand the limited treatment options for brain tumor patients. However, for far too many, access to the full spectrum of treatment options, which can mean the difference between a positive outcome with good quality of life and a poor prognosis, is a challenge.

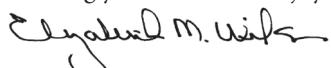
Too frequently we hear from brain tumor patients or their caregivers that, 'If I knew then what I know now, I might have made different decisions.' This is because when someone learns they have a brain tumor, they are overwhelmed – by the diagnosis itself, by a new and difficult vocabulary, and by the urgency to make difficult decisions about their course of treatment.

The ABTA is working tirelessly to ensure that patients and families have the information and resources they need at the point of diagnosis and throughout the trajectory of the disease. Our recently launched ABTA CommYOUtity, a nationwide volunteer network, is just one of the ways we are extending our reach to more people, in more places and in more meaningful ways. To learn more, go to www.abta.org/commYOUtity.

In this issue of *Headlines*, we've included the perspectives of nationally recognized experts on the current state of discovery science, clinical trials and new drug development, as well as the road to personalized medicine for brain tumor patients. We also feature a caregiver who shares the impact a brain tumor diagnosis had on his family and the competing demands of balancing work, family and his own needs. We hope you find our redesign, and the inclusion of more tips, trends and questions to ask your doctor helpful. Let us know what you think by emailing us at abtacares@abta.org.

In this the season of counting blessings, know that the ABTA counts among ours the generous support we receive from so many throughout the year. Your support allows the ABTA to fund researchers and research projects with the potential to further the field and inform the educational programs and resources we provide to those impacted by a brain tumor diagnosis. Learn just how impactful your support of the ABTA mission is from one of our funded researchers at www.abta.org/Derek.

Wishing you comfort and joy this Holiday Season,



Elizabeth M. Wilson, MNA
President & CEO

Headlines

VOLUME 41, NUMBER 4

Our mission: The mission of the American Brain Tumor Association is to advance the understanding and treatment of brain tumors with the goals of improving, extending and, ultimately, saving the lives of those impacted by a brain tumor diagnosis.

We do this through interactions and engagements with brain tumor patients and their families, collaborations with allied groups and organizations, and the funding of brain tumor research.

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Through the Eyes of a Caregiver

A father, a husband, a family's story...



Five-year-old Lindyn and eight-year-old Lauryn left for school filled with the excitement and anticipation only little princesses can muster knowing they soon would be leaving for the most magical place on earth—Disney World. Unfortunately, their family vacation, and the world as they knew it, would abruptly change.

"Thinking back on that day, I remember trying to pick the right words to explain what's going on with Mommy, why we weren't going to Disney World" said Brian Fatula. "I could see that they were terrified, and I was trying my best to share what I felt was appropriate for their age so it wasn't too big, too much, but at the same time, answer their questions the best that I could."

Taking on the role of caregiver for his wife Katy who was diagnosed with an oligodendrolioma brain tumor was something Brian never expected. He cherished his role as a loving father and husband, and now the role of caregiver was not only unexpected but foreign territory that he quickly needed to navigate.

Many individuals like Brian who are suddenly thrust into caregiving for a spouse or loved one feel the added responsibilities can challenge them in ways they never imagined.

"Caregivers often feel the stress of the immediacy of becoming an expert not only in attending to their loved one's physical needs, but also in managing the details and communication with doctors, pharmacists, insurance companies, employers and especially family," explained Mary Lovely, PhD, RN, CNRN, senior advisor, American Brain Tumor Association. "Brian found he quickly needed to balance his daughters' needs with his wife's needs, and often, caregivers like him ignore their own needs in the midst of caring for others."

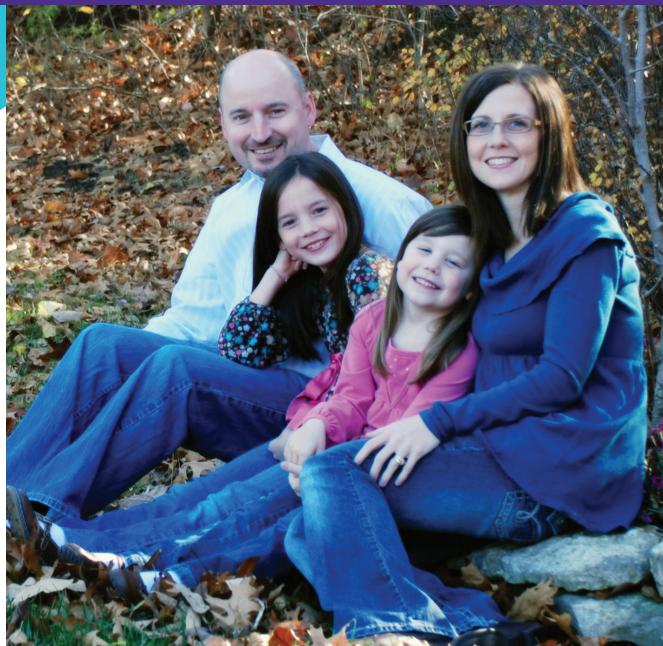
While it took Brian nearly a year before he felt ready to do something just for himself, he still can't shake the feelings he had when he left the house that day.

"I decided I was going to go out and play golf, but I had nothing but guilt on my mind. All I kept thinking was, I'm out here trying to enjoy myself and my wife's at home with someone else taking care of her," Brian recalls. "That day was my first step in realizing that I too needed some help and support. If you don't take care of yourself first, then you can't take care of somebody else to the best of your ability."

The challenges of balancing work, family, and a caregiver's own needs, while caring for someone else and fulfilling day-to-day responsibilities are unique to each caregiver.

"Because everyone's situation is different, we encourage caregivers to call or email us anytime with questions so we can help them find information or resources to help them cope," added Lovely. "Caregivers are not alone in this journey. The ABTA is here to help." 

 To learn more about Brian's story or for more information, tips and resources for caregivers, go to www.abta.org/headlines



The Fatula Family: Brian, Lauryn, Lindyn and Katy the day before Katy's craniotomy 3 years ago.

Tips for Talking with Children

- Describe the illness and treatment. Use appropriate language for your children's age; consider picture books and read to younger children.
- Practice your explanations. Your conversation will be most effective if you know beforehand what you are going to say and the words you will use.
- There is no way to predict a child's response. They may surprise you with their insight, or they may have their own ideas and explanations. Avoid over-correcting them and allow them to offer their own explanations as well.
- Young children may think they (or someone else) may have done something to cause the brain tumor. If this idea comes up when talking with your children, reassure them that no one causes a brain tumor. If your children do not raise the idea, then you should avoid talking about fault, as it could lead them to wonder if, in fact, they did have a role.

The ABTA's toll free CareLine **800-886-ABTA (2282)**, is answered by health care professionals, Monday-Friday 8 a.m.– 5:30 p.m. CT, or email ABTAcares@abta.org.

The Road to Personalized Medicine

Where will it Lead Brain Tumor Treatment?

Personalized medicine, sometimes called precision medicine, is a developing area that utilizes an individual's genetic information to help inform diagnosis and treatment decisions.

"Brain tumors arise because there are a lot of genetic changes that occur that cause cells to divide and grow. We're now able to better identify and understand these genetic changes, and we also know that these changes can be different from patient to patient," explains Patrick Wen, MD, professor of neurology, Harvard Medical School, director of the Center for Neuro-Oncology, Dana-Farber Cancer Institute. "That's why we're studying an array of 'personalized medicine' options as an alternative to a one-size-fits-all approach."

The Road Most Traveled: Standard Treatment

For the most common, grade IV, malignant primary tumor, glioblastoma (GBM), today's standard treatment begins with surgical removal of as much of the tumor as possible. Surgery is followed by six weeks of radiation therapy and chemotherapy treatment with the drug Temodar®. Patients usually continue chemotherapy for six months.



Far left: Dr. Wen

"Today's standard therapy involves giving the same drug to everybody hoping that it will work," said Dr. Wen. "With new drugs called targeted molecular therapies, we know that you have to give the right drugs to the right patients. We need to identify the molecular changes in each patient's tumor and then give the drug that is more likely to be effective based on what we know about the molecular changes that have been identified."

A New Direction: Genotyping

How does a physician identify these molecular changes? A test called genotyping can reveal the specific mutations within a patient's tumor from tissue that is collected at the time of surgery and is then frozen and/or embedded in paraffin blocks. Unlike genetic testing which seeks to confirm or rule out genetic conditions that potentially run

in families, genotyping aims to identify specific mutations within the tumor.

"We want more patients to be aware that genotyping is an option that they should discuss with their physician at the time of diagnosis," said Elizabeth M. Wilson, MNA, president and CEO, American Brain Tumor Association. "Having these discussions early can help inform treatment decisions so as many options as possible remain open to them throughout the trajectory of the disease."

Dr. Wen agrees that patients need to advocate for themselves. "If you want your tumor genotyped, it is important to talk with the surgeon about this in advance to make sure that enough tissue is removed to make this testing possible," explained Dr. Wen. "It's a test that is becoming available at more centers around the country."

Why Consider Genotyping?

Genotyping informs what treatments may be most effective as well as treatments that would not offer any benefit. For example, genotyping can reveal if a patient has a protein called methyl-guanine-methyl-transferase or MGMT. When present in high levels, MGMT reduces the therapeutic effect of the chemotherapy treatment Temodar®, so the side effects of chemotherapy may outweigh any potential benefit.

Genotyping also can determine if a patient has the IDH1 mutation which can be a predictor of a patient's prognosis, and there are now drugs being developed that target this specific mutation.

Targeted Molecular Therapies

Drugs that are designed to "target" or interrupt specific tumor mutations that cause tumors to grow are called targeted molecular therapies. Since brain tumors don't develop the same way in every individual, targeted molecular therapies offer brain tumor patients treatments that are "personalized" to their specific tumor and offer the potential for fewer side effects than standard treatment with radiation and chemotherapy.

Examples of targeted molecular therapies in development to treat brain tumors include: PI3 kinase inhibitors, vaccines that are based on the patient's specific tumor mutations, drugs used to target the epidermal growth factor (EGFR), and many other receptors found in brain tumors.

Currently, targeted molecular therapies are only available to brain tumor patients through **clinical trials**—research studies used to determine if the new therapy is safe and effective. Clinical trials are particularly important to brain tumor patients because they offer treatment options, like targeted molecular therapies or vaccines that would otherwise not be available.

According to Dr. Wen, one of the biggest challenges to advancing new brain tumor treatments is the small number of patients who participate in clinical trials.

He estimates that it is less than 10 percent and identifies low participation as one aspect hindering the progress for new brain tumor treatments.

"Treatment for melanoma used to be like glioblastomas where there were few options and the prognosis wasn't promising," said Dr. Wen. "Suddenly genetic mutations were found in melanoma patients and now there are several drugs to treat these mutations, and melanoma patients are seeing good responses. I am hopeful that we can do the same for brain tumors if we can interest more patients in participating in clinical trials."

Wilson adds, "Knowledge is power, and that is especially true when it comes to treating brain tumors. Once diagnosed, the sooner patients understand their tumor and their treatment options, the more equipped they will be to discuss those options with their physicians and ensure they are receiving the most optimal course of treatment, whether it's standard of care, enrollment in a clinical trial or a combination of both." 

What Should I Ask My Doctor?

- ✓ What will genotyping tell us about my brain tumor?
- ✓ When is the optimal time to have this testing?
- ✓ How likely is it that genotyping will reveal a mutation for which there is a targeted treatment available for me?
- ✓ If I decide to have my tumor genotyped, are there any additional tests I need to have performed?
- ✓ How can I make sure enough tissue is removed to make this testing possible?
- ✓ How long does it take to get the results?
- ✓ Will my insurance cover the test?
- ✓ Is tumor genotyping conducted at this hospital?
- ✓ If it isn't available here, can I be referred to a medical center where the test is performed or is there a commercial test that can be used?

Make an Impact Today

Every day, 500 people in the U.S. are diagnosed with a brain tumor, and your support helps the ABTA make an impact in the lives of those living with this devastating disease and those dedicated to brain tumor research.

Young investigators like Derek Wainwright, PhD, assistant professor of Neurological Surgery, Northwestern University Feinberg School of Medicine, need ABTA funding to gain a foothold in the brain tumor research community, and your donations ensure that the best and brightest minds are devoted to pursuing the answers brain tumor patients and families desperately seek.

"The ABTA supported my research and career as a young scientist, educator and advocate for individuals with brain tumors. It's a very exciting time given that DNA sequencing, large-scale gene profiling and immunotherapeutic engineering are expanding our capabilities to personalize medicine and uncover new modalities for improving the health and survival of patients with brain cancer."

— DEREK WAINWRIGHT, PH.D., ABTA RESEARCH GRANT RECIPIENT



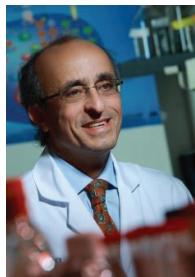
On behalf of the thousands of brain tumor patients and caregivers whose lives are better due to your commitment, and the researchers who are able to move their ideas forward because of your support, the ABTA asks that you donate today.

Experience the impact you can make by watching a video of Derek's story and supporting researchers like him. Visit abta.org/Derek or use the envelope in this newsletter to make a donation.

From Concept to Clinic

The Role of Discovery Science in New Drug Development

The process of discovering a new concept in the lab and translating it to an FDA-approved drug treatment takes considerable time and significant funding.



"The costs, soup to nuts, can be astronomical," said John Laterra, MD, professor of Neurology and Oncology, and director of Neuro-Oncology, Johns Hopkins. "And the discovery process takes time and must accommodate false starts and redirections before a promising concept leads to a potential new compound for clinical testing."

"Remarkably, for every 5,000 compounds being tested pre-clinically in the laboratory, only about five percent enter the first phase of clinical testing," stated Dr. Laterra.

Discovery Science

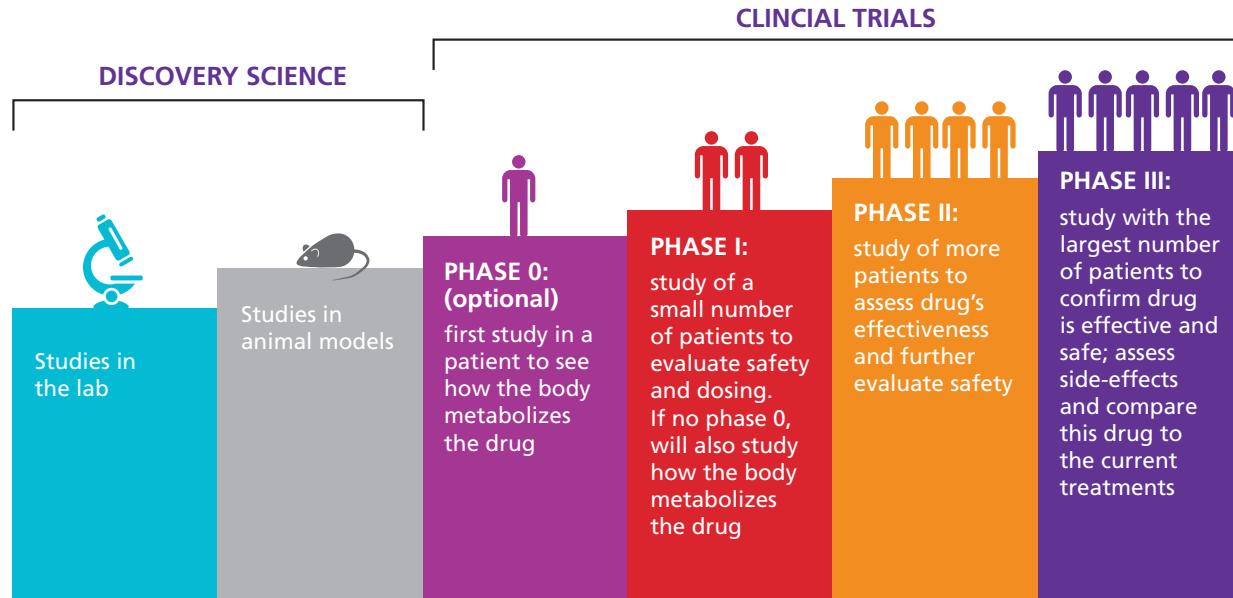
One of the first steps of drug development takes place during a preclinical phase called discovery science where researchers act as keen detectives. They explore molecular pathways that drive uncontrolled growth of brain tumor cells.

"Discovery science is where new drug development begins and is critically important to furthering our understanding of the causes of brain tumors. It establishes the foundation for drug development directed at ultimately finding new treatments," said Dr. Laterra. "That's why support for this phase of research is so important."

Once researchers have a target for what they understand to be causing tumor growth, they begin working in the lab to identify strategies and molecular compounds (drugs) that will inhibit the target and its related functions in cancer cells. Overcoming scientific obstacles are challenging enough. However, simple economics can prevent a promising new concept and compound from moving forward. As Dr. Laterra explains, competition for this type of funding is high, and funding for discovery science is historically low. In some cases, researchers do not secure additional funding to keep their promising studies going.

ABTA Discovery Grants

Recognizing the need to increase funding in this area, the ABTA launched its Discovery Grants program in 2010 to support researchers pursuing high-risk, high-impact projects with the potential to change current diagnostic or treatment paradigms. This year alone, the ABTA awarded \$400,000 in grants to further discovery science.



In just three years, ABTA Discovery Grant funding has aided the development of two new brain tumor drugs that target malignant gliomas such as glioblastoma (GBM).

"We are proud of the early successes of this program and remain committed to funding brilliant, innovative researchers because the need for discovery science has never been greater," said Elizabeth M. Wilson, MNA, president and CEO, American Brain Tumor Association. "These researchers are on the front lines of identifying new and more effective diagnostic and treatment options and they need the funding to move these ideas forward."

Recently OLIG2 inhibitor research—a concept initially funded with an ABTA Discovery Grant—received additional funding to advance it through the preclinical phase of development. To date, the ABTA's \$2 million investment in discovery research funding has led to \$11 million in follow-on investments and facilitated the development of two new "first-in-class" drugs for the neuro-oncology community.

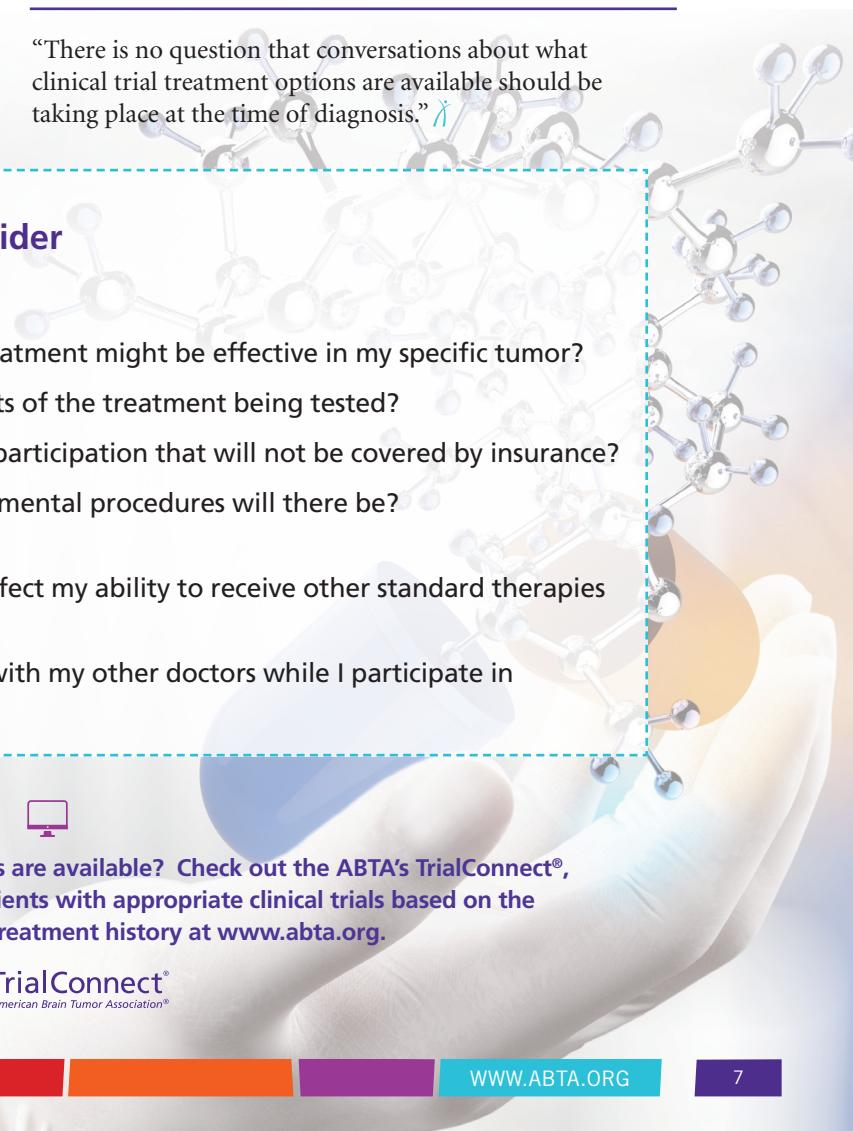
"Our efforts with OLIG2 inhibitors wouldn't be possible without the funding we received early on from the American Brain Tumor Association," said Santosh Kesari, MD, PhD, professor of Neurosciences at UC San Diego School of

Medicine and director of Neuro-oncology at Moores UCSD Cancer Center. "We appreciate the ABTA's support and share their pride in seeing this new therapeutic approach move closer to clinical trials."

Clinical Trials

After thousands of drugs are narrowed down in the discovery science phase, only a select few will reach Phase I clinical trials. This is the phase where researchers evaluate the drug's safety, metabolism and tissue distributions in patients. If the Phase I trial confirms safety and identifies an adequate dosing scheme, the drug will progress to the next phases focusing more on establishing anti-tumor effects with the goal of completing three phases of clinical trials before it goes before the FDA for approval.

"The only way that future brain tumor patients are going to have better outcomes is for current patients to participate in the drug discovery process," added Dr. Laterra.

"There is no question that conversations about what clinical trial treatment options are available should be taking place at the time of diagnosis." 

Clinical Trials: Questions to Consider

- ✓ What is the purpose of the trial?
- ✓ Why do researchers believe this new treatment might be effective in my specific tumor?
- ✓ What are the known possible side effects of the treatment being tested?
- ✓ Are there expenses associated with my participation that will not be covered by insurance?
- ✓ How many treatments and other experimental procedures will there be? How long will each one take?
- ✓ How will my participation in this trial affect my ability to receive other standard therapies or clinical trial therapy in the future?
- ✓ How will the study doctors coordinate with my other doctors while I participate in the study?



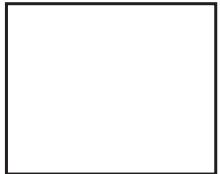
Want to learn about what clinical trials are available? Check out the ABTA's TrialConnect®, a free service that links brain tumor patients with appropriate clinical trials based on the patient's tumor type and treatment history at www.abta.org.





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LATE-BREAKING NEWS

As this newsletter was going to print, phase III clinical trial data was released showing a device (Optune™) that delivers Tumor Treating Fields (TTFields) in combination with standard-of-care Temozolamide chemotherapy extended both progression-free survival and overall survival compared to Temozolamide alone in patients with newly diagnosed glioblastoma (GBM). Currently, Optune™ is only FDA approved for treatment in the U.S. for patients with recurrent GBM. For more information, call ABTA's toll-free CareLine 800-886-2282.



HARNESSING THE POWER OF "BIG DATA"

The term "big data" is a buzzword used across many industries to label large amounts of information or "data sets" collected for analysis. There is increasing interest in "big data" in healthcare to analyze patterns, abnormalities, and trends with the potential to identify new targets and ultimately new treatments. One of the newest projects—beginning with other cancers and moving toward brain tumors—will be the use of IBM's big data computer named Watson to organize the information in electronic medical records.

Breakthrough Opportunities

Walks and Runs

- | | |
|----------|--------------------------------|
| March 21 | BT5K Tampa Bay
Tampa, FL |
| April 11 | BT5K Los Angeles
Pomona, CA |
| April 26 | BT5K Chicago
Chicago, IL |
| May 2 | BT5K Michigan
Milford, MI |
| May 16 | BT5K Spokane
Spokane, WA |
| June 13 | BT5K Columbus
Columbus, OH |



Webinars (Visit www.abta.org for a full listing)

- | | |
|------------------|---|
| January 7, 2015 | Financial and Legal Resources for Brain Tumor Patients |
| January 21, 2015 | Brain Tumors 101 |
| April 23, 2015 | Integrative Oncology and Nutrition for Brain Tumor Patients |

Patient & Caregiver Meetings

Educational meetings covering a variety of brain tumor topics will be held in select locations across the country in 2015. Look for more updates at www.abta.org.