

NEUROtransmitter *Communicating our message.*

Pediatric Brain Tumor National and International Clinical Trials Consortia: an Overview

Feature Article by Dr. Roger J. Packer

As the care of children with brain tumors evolves, there is recognition that despite advances in surgery, chemotherapy and radiation therapy, there are clear needs to explore other options and to create mechanisms where new therapeutic approaches can be assessed carefully and expeditiously. Over the past 2 decades, multiple clinical trials consortia have been developed to meet these needs. These consortia were developed to supplement activities of well-established clinical trials organizations, such as the Children’s Oncology Group (COG) and the International Society of Pediatric Oncology (SIOP). It was envisioned that these smaller consortia would be comprised of institutions that had the expertise and infrastructure to safely perform innovative, often challenging trials. Once new approaches and agents trials were proven to be of potential benefit, they could be prospectively evaluated in larger studies attempting to determine if such new innovative therapies were superior to or equal to but causing less toxicity than conventional means of management. Subsequent prospective Phase 3 studies could be performed through the COG or similar groups. The new consortia were also designed to discover important biomarkers, but were not designed to focus on basic brain tumor-related research. As these consortia have evolved, even though they continue to focus on phase I (dose findings) and phase II (determination of efficacy) studies, they undertook some trials aimed at changing management for diseases for which there was not an accepted reasonable standard of care. This was especially true for patients with diffuse intrinsic pontine gliomas (DIPGs). Although each of these consortia had somewhat different initial objectives, over time, as these consortia developed, the differences between each individual study group blurred. Because of this blurring of differences between the different consortia, competing studies are frequently open for the same population of patients, making it somewhat confusing for both physicians and families to determine the optimum approach for a given child. However, having multiple studies open is critical to afford patients the most innovative in treatment, hopefully within geographic reach of the child and family, and to make progress most rapidly.

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Be AMYazing! Reston Youth League

The Be AMYazing ! Reston Youth League Triathlon was founded in 2008 by Amy’s Amigos. A large group of 12-year-olds, Amy’s Amigos, offered support for Amy Boyle, a classmate battling brain cancer. In 2011 after Amy’s death from DIPG, Amy’s Amigos held the first Reston triathlon for children.

The event date is planned for May 10, but may change to June depending on the current status of COVID-19. Registration is open and the site for updates: www.restonyouthtri.org

CBTF RAISES FUNDS FOR RESEARCH

Funds raised benefit pediatric brain tumor research and other CBTF programs

The Childhood Brain Tumor Foundation

Our mission is to support and fund basic science or clinical research for childhood brain tumors. We are dedicated to heightening public awareness of this devastating disease, and improving the quality of life for those that it affects by funding vital research initiatives.



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Our new website is open!

Clinical Trials Consortia

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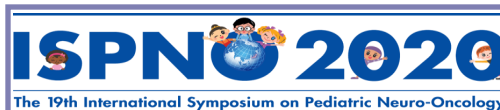
Both Children's National and its regional partners and Johns Hopkins Hospital have worked diligently to make these novel consortia studies available in the Washington-Baltimore region. Increasingly studies have focused on molecular-targeted therapy and immunotherapy, although new radiation therapy and surgery-oriented studies are also being performed. The Brain Tumor Program at Children's National works closely with its partners including Pediatric Specialists of Northern Virginia (PSV), Inova Fairfax Hospital, The King's Daughters Hospital of Norfolk, VA, the University of Virginia at Charlottesville, VA, Richmond Children's Hospital (VCU), Carilion Hospital, the National Cancer Institute and the joint Johns Hopkins and Children's National Proton Beam Unit at Sibley Hospital and has developed a regional network to support Consortium involvement and to make studies more available to children in Washington, DC, Maryland, Virginia and West Virginia. Children's National runs a virtual brain tumor board between the above hospitals and others including the University of Maryland in Baltimore, Geisinger Medical Center in Pennsylvania, Yale University, Pittsburgh Children's Hospital and Downstate Medical Center in New York to facilitate dissemination of information concerning availability of innovative therapies.

These consortia include:

Pediatric Brain Tumor Consortium - PBTC

In April of 1999, the Children's National Hospital (then the Children's National Medical Center) was named as one of the 9 initial institutions in the United States selected for membership in the then newly formed Pediatric Brain Tumor Consortium (PBTC). This Consortium, supported by the National Institute of Health and the National Cancer Institute, was developed to perform innovative, technologically challenging studies for children with brain tumors; studies designed both to improve survival and quality-of-life. The institutions were chosen after a competitive review of their past experience in the care of children with brain tumors, the depth of their pediatric program, the clinical resources of the institution and their ability to perform innovative research. This Consortium has been in continuous operation over the past 20 years. Every 2 to 3 years institutions are re-evaluated for their suitability to be part of the Consortium and their contributions to the Consortium, including scientific contributions and patient accruals. Children's National is proud to be one of only 3 institutions (the others are St. Jude's and Texas Children's Hospital) that have been continuous full members since the inception of the PBTC. Children's National

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The Childhood Brain Tumor Foundation is a SILVER sponsor for the 19th International Symposium on Pediatric Neuro-Oncology. Meeting date has changed and the ISPNO will now be held, **December 11 to December 13, 2020** in Karuizawa, Nagano, Japan. As always the ISPNO brings together clinicians, researchers, allied health professionals, and experts.

If you are interested in learning more about the Childhood Brain Tumor Foundation, Inc.,

Please contact: **E-MAIL:** cbtf@childhoodbraintumor.org or jeannyoung@childhoodbraintumor.org (**E-mail preferred** due to high volume of robo-calls)

TELEPHONE: 877-217-4166 or 301-515-2900

Volunteers welcome!

BE AMYAZING! ...MORE PHOTOS



2007: Amy enjoying blackjack with family members and Dr. Packer.



Upcoming Events 2020

Childhood Brain Tumor Conference: Education and Updates:
(TBD—possible changes due to COVID-19)
Our website will have updates and other information.

CBTF Party 2020

Glenview Mansion, Rockville—Saturday, December 5, 2020

Fundraising events and donations are significant to our ability to fund excellent research. Thank you for your support!

Thank you so much!

Be AMYazing, Reston Youth League
FD Associates, Inc.
Roger and Bashi Packer
John Paul II School

Special thanks from CBTF to the research and medical community for their dedication!

Donations Welcome

Annual Superheroes 5K and Kids Run 2020 Sligo Creek Pkwy, Silver Spring.

Due to COVID-19 concerns the April 19 date was cancelled. A **new date will be determined** and the online race registration page and the new CBTF website will be have updated information. The registration/donation page is open.

<https://tinyurl.com/5kCBTF20>



CBTF Sponsorship:

2020 -19th International Symposium on Pediatric Neuro-Oncology

2019-2020 Society for Neuro-Oncology, International, Sub-Saharan Africa

Consortia.....

has led many studies within the Consortium and still has a major leadership role. Dr. Roger J. Packer, Senior Vice President of the Center for Neuroscience and Behavioral Medicine and Director of the Brain Tumor Institute at Children's National Hospital, was the initial Principal Investigator for Children's National to the Consortium. As of 2019, Dr. Eugene Hwang took over the role of Site Principal Investigator at Children's National, but Dr. Packer remains active in Consortium activities as co-principal site investigator, member of its Senior Advisory Committee, and leads various committees within the PBTC.

Pediatric (Pacific) Neuro-Oncology Consortium – PNO

Approximately 15 years ago the Pediatric (Pacific) Neuro-Oncology Consortium (PNO) was developed by the University of California at San Francisco's brain tumor program, involving only Pacific Coast sites to also perform innovative, often technologically challenging studies for children with brain tumors. PNO is primarily supported by industry and philanthropy and has no direct NIH funding. It is a very facile Consortium that has been able to open and perform studies quickly with a major focus on studies utilizing innovative molecular-targeted therapies and, more recently, immunotherapy. It also strives to be involved in technologically challenging studies. Children's National Hospital became the first East Coast site of the Consortium when the Consortium decided to expand nationally approximately 10 years ago. Dr. Packer served as Children's National's first Principal Investigator and since 2018 has moved to a co-Principal Investigator role, as Dr. Lindsay Kilburn has taken over the Principal Investigator role. Johns Hopkins has subsequently joined PNO under the leadership of Dr. Kenneth Cohen. PNO also works closely with the Childhood Brain Tumor Tissue Consortium (CBTTC) which now has formally merged some activities with PNO. The CBTTC, headquartered at Children's Hospital of Philadelphia, is an international brain tumor repository and registry which molecularly characterizes all tissues received and immediately puts this information in the "cloud", to make the information available to investigators. Dr. Brian Rood serves as the Principal Investigator for the CBTTC efforts at Children's National and Dr. Javad Nazarian, also at Children's National, has served as the Scientific Director of the CBTTC.

Department of Defense Neurofibromatosis Clinical Trials Consortium - DoDNFCTC

This Consortium was developed by the Department of Defense to perform innovative trials for children and young adults with neurofibromatosis type 1, neurofibromatosis type 2 and Schwannomatosis. The Consortium opened approximately 15 years ago and Children's National has been a member of the DoDNFCTC continuously since. Johns Hopkins Hospital, under the direction of Dr. Jaishri Blakeley, entered as a subsite of Children's National five years ago. Dr. Packer was the first national Principal Investigator of the consortium and held that post for the initial 10 years of the DoDNFCTC. Due to term limits Dr. Packer, relinquished the national Principal Investigator role, but remains the Children's National site PI. Dr. Miriam Bornhorst, Director of Children's

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Please support the Childhood Brain Tumor Foundation

Visit our [GIVE ONLINE](#) donation button:

<https://www.givedirect.org/donate/?cid=1605>

Be part of the solution in helping fund vital research initiatives cure childhood brain tumors!

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RESEARCH SUMMARIES, Give2Charity

National Clinical Neurofibromatosis Program, has taken increased responsibilities for Children's National role in the DoDNFCTC. The Consortium has completed multiple clinical trials for children with NF and both plexiform neurofibromas and visual pathway gliomas. Dr. Blakeley has undertaken leadership roles in studies evaluating new approaches for NF2-associated acoustic schwannomas and developing studies for patients with schwannomatosis. Working with the Sarcoma Alliance, the DoDNFCTC has also undertaken studies for children and adults with malignant peripheral nerve sheath tumors (MPNSTs), Dr. Aerang Kim from Children's National has taken a major leadership role in these MPNSTs efforts, working closely with Dr. Brigitte Widemann, Director of the Pediatric Branch of the National Cancer Institute.

Remission Alliance

Children's National has worked closely with Duke University and the University of Florida through the Remission Alliance to develop immunotherapy protocols for children with both recurrent and newly diagnosed high-grade brain tumors; both medulloblastomas and high-grade gliomas. The Alliance now numbers 12 institutions, performing studies in both adults and children. Dr. Duane Mitchell is the national PI of the Remission Alliance. Dr. Mitchell has an adjunct appointment at Children's National and works closely with Dr. Eugene Hwang, who is the site PI at Children's National for the Remission Alliance. Dr. Packer is site co-PI.

CONNECT

This is a new international Consortium being coordinated presently at Cincinnati Children's Medical Center. Dr. Packer is site PI at Children's National for CONNECT, which also strives to do innovative and technologically challenging studies for children with brain tumors. It also works very closely with industry to perform these studies. One of the earliest studies begun by CONNECT is being chaired by Dr. Eugene Hwang from Children's National. To date CONNECT has primarily focused on those children with high-grade brain tumors, including diffuse intrinsic pontine gliomas and medulloblastomas.

Children's Oncology Group (COG) Phase I Consortium

Children's National and Johns Hopkins both have been long standing member of the Children's Oncology Group (COG). This is an international group of over 100 institutions and has many open studies for children with newly diagnosed disease. In addition through the **COG Phase I Consortium (DVL)**, of which Children's National and Johns Hopkins are members, molecular-targeted studies, as well as innovative immunotherapy studies, are being performed.

Other Consortia

Children's National and Johns Hopkins both are part of the Head Start Consortia, with Dr. Eugene Hwang being the site PI, at Children's National and Dr. Kenneth Cohen holding a similar role at Johns Hopkins. This Consortium predominantly does studies for infants and young children with medulloblastoma, utilizing high-dose chemotherapy.

Children's National is also working closely with St. Jude's and has been an active site for its molecularly-stratified based studies for newly diagnosed patients with medulloblastoma and for children with both newly diagnosed and recurrent atypical teratoid/rhabdoid tumors.

Children's National and Johns Hopkins also have multiple industry trials open through industry-sponsored and coordinated consortia. Both are working closely with Novartis on molecular-targeted studies for children with BRAF-mutated high-grade and low-grade gliomas. Johns Hopkins' program is working with industry on sponsored trials with BMS, Epizyme and Esai.

Roger J. Packer, MD, Senior Vice-President, Center for Neuroscience and Behavioral Medicine, Director, Brain Tumor Institute, Director, Gilbert Family Neurofibromatosis Institute

The Childhood Brain Tumor Foundation is dedicated to funding research for all pediatric brain tumor types.



Please contact us if you have any questions:
cbtf@childhoodbraintumor.org

To make a donation to our cause, visit our secure website:
www.childhoodbraintumor.org



HELPFUL RESOURCE LINKS

Thoughts Can Fuel Some Deadly Brain Cancers (paper in Cell), Michelle Monje

"The discovery of a link between tumor growth and brain activity "has opened up a window into potential therapeutic interventions," says Bachelor

Follow the link for more information:
<http://www.npr.org/blogs/health/2015/04/23/401723235/thoughts-can-fuel-some-deadly-brain-cancers>

GRACIOUS GIVING



Maryland Charity Campaign

The Childhood Brain Tumor Foundation participates in the **Combined Federal Campaign (CFC)** and **Maryland Charities**.

Gift Matching Opportunities

Many companies offer a matching gifts program to support charitable organizations.

Your human resources department can tell you if such a program exists at your company. Ask them about the form that can be sent to the Childhood Brain Tumor Foundation reporting a contribution (donation or event contribution). The form states that they will match your contribution.

We return the form to the employer with the proper acknowledgment and information required.



Thank you to our friends who donated through workplace charitable giving campaigns this year, inclusive of the CFC.

The Childhood Brain Tumor Foundation, friends and families are very appreciative of your support. (National) CFC **12035**

Charity Campaign and other independent campaigns.

Campaign donations may be made for the United Way (**UW**) through the "donor option" or "donor choice." Please check with your employer in reference to **UW** campaigns. You may write us in.

Thank you donors!

Vehicle Donation Program



CBTF accepts vehicle donations. Help make a difference, donate online or call 877-999-8322 and designate the Childhood Brain Tumor Foundation as your charity of choice.

The service is totally free and includes convenient pick-up of your car, truck, or RV anywhere in the U.S.

There are two easy ways to make a donation:

GIVE ONLINE: Visit our secure website: www.childhoodbraintumor.org
Go to our **Support** page and click on the **Give2Charity** button.
(Accepts Discover, MC, VISA, and AE).

BY CHECK: Made out to **CBTF**
Send to: **CBTF**
20312 Watkins Meadow Drive
Germantown, MD 20874

THANK YOU FOR YOUR SUPPORT!
Your contributions make it possible for CBTF to fulfill its mission.

Stock Donations

If you would like to make a stock donation, contact us: cbtf@childhoodbraintumor.org

Our treasurer will provide you with the necessary details to proceed with your donation.

Thank you.

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together, reaching for a cure!

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Our new website is open!

ANNUAL Superheroes 5 K and Kids' Run 2020, Shigo Creek Pkwy/ Potomac River Running website, for donations as we determine the new date

Registration remains open on the website, for donations as we determine the new date

To be rescheduled

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