Winter 2023

$1M Raised Using Humor to Inspire Hope for Children with Brain Tumors

Meryl Witmer Honored at the Annual "No Laughing Matter" Comedy Night Fundraiser

The fifth annual "No Laughing Matter" comedy night took place on October 12, setting a new fundraising record with $1,000,000 raised to support pediatric brain tumor research at Weill Cornell Medicine. Sponsored by Aquarian Holdings and organized by the Children's Brain Tumor Project Foundation, the event unfolded at the esteemed New York Athletic Club, serving as a crucial platform for raising funds that directly benefit the groundbreaking research initiatives led by Dr. Mark Souweidane and Dr. Jeffrey Greenfield at the Children's Brain Tumor Project (CBTP) lab.

"We are so grateful to the board of directors at the Children's Brain Tumor Project Foundation, our dinner chair Tara Lipton, our honoree Meryl Witmer, our generous donors and sponsors, and the incredible comedic talent who made this event such a tremendous success," said Dr. Jeffrey Greenfield. "We rely on the success of this event not only to sustain existing research, but also to expand our efforts, hastening the discovery of cures and their transition to clinical applications."

In attendance were several families directly affected by pediatric brain tumor diagnoses, including the courageous Liam Berg (8 years old), who took the stage to share inspirational words, and the talented Kai Brown Coley (10 years old), who showcased his musical prowess on the baby grand piano during the cocktail hour.

Liz Laugh Love Award Recipient Meryl Witmer

A significant highlight of the night was the presentation of the "Liz Laugh Love" award to Meryl Witmer, a dedicated advocate for children with brain tumors. Witmer's commitment stems from her own personal experience, having navigated her son Andrew's medulloblastoma diagnosis many years ago. Andrew, a graduate of Princeton University in 2020 with a degree in mechanical and aerospace engineering, now works in the Washington D.C. area, with his professional success a testament to the great care he received at a young age.

Ms. Witmer has shown generous support for many clinical trials and research advancements, including Dr. Mark Souweidane's novel work using convection-enhanced delivery in children with brain tumors. Her door is always open to families who face this frightening diagnosis, and she demonstrates unwavering commitment to this cause.

The "Liz Laugh Love" award, named in memory of Elizabeth (Liz) Minter, is a prestigious honor that acknowledges individuals for their exemplary advocacy within the pediatric brain tumor community. Liz Minter, celebrated for her boundless love and contagious laughter, initiated a powerful fundraising effort before her passing in 2012. This initiative played a pivotal role in establishing the foundation for the Weill Cornell Medicine Children's Brain Tumor Project. Notable recipients of this esteemed award in previous years have included Mike and Emmie Minter, Tim Michels, and Veronica Marsano.

The Lineup

An all-star comedy lineup donated time and talent to the laugh-out-loud fundraiser on October 12, to help raise research funding for rare and inoperable brain tumors at Weill Cornell Medicine. Comedians included Michael Cruz Kayne, Chase O'Donnell, Carmen Lynch, Michael Kosta, and the Improvised Shakespeare Company.

The Research Appeal

Auctioneer Kenny Polcari, a managing partner at Kace Capital Advisory and market analyst on CNBC, has a close friend who lost her son, Lucas, to an inoperable brain tumor called DIPG. After showing a beautiful video featuring Lucas' heartbreaking story, Kenny proceeded to encourage generous donations, ranging from $50,000 to $500, to meet the fundraising goal for the research appeal. Through Kenny's heartfelt appeal and the shared empathy of those present, the event successfully garnered the financial support necessary for advancing critical research in the ongoing battle against pediatric brain tumors.

Left to Right: Kari Coley and her son, Kai (who performed piano); Dr. Mark Souweidane and Dr. Jeffrey Greenfield; Andrew Witmer with mom Meryl Witmer, Liz Laugh Love Award Recipient; Comedian Michael Kosta; Dinner Chair, Tara Lipton

Paper on Post-Mortem Tissue Donation Published in Pediatric Neurology

Designated as a Center of Excellence by Gift from a Child, the rapid autopsy donation program at Weill Cornell Medicine worked in collaboration with Memorial Sloan Kettering Cancer Center to creatively navigate the challenges posed by the pandemic to fulfill parents' desires to donate tumor tissue.

The acquisition of postmortem tissue from pediatric oncology patients is crucial for research and serves as a meaningful way for grieving families to find solace. Since 2019, the national Gift from a Child program has made significant strides in gathering postmortem tissue from pediatric patients with central nervous system tumors to advance research. However, this progress faced jeopardy with the onset of the pandemic, which led to the suspension of many autopsy programs.

In a recently published peer-reviewed paper, our team conducted a retrospective review of autopsies involving four patients treated at Memorial Sloan Kettering Cancer Center. These patients underwent postmortem examination at Weill Cornell Medicine between June 2020 and March 2021. The paper explores the unique challenges and innovative strategies employed by our collaborative team during this period, highlighting the importance of maintaining momentum in postmortem tissue donation and research efforts, even in the face of unprecedented obstacles.

Team CBTP Finishes Strong at the TCS New York City Marathon

$70,000 Raised to Benefit Pediatric Brain Tumor Research at Weill Cornell Medicine

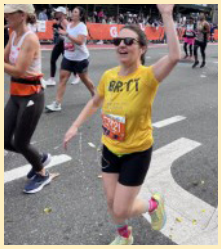
The CBTP Foundation is an Official Charity Partner for the TCS New York City Marathon. On Sunday, November 5, our team of 16 athletes hit the streets of New York City, running 26.2 miles through all five boroughs in the world's biggest and boldest marathon. If you are interested in running a marathon or half marathon in New York City, visit **cbtpfoundation.org/run**.


Brooke

"Running the NYC Marathon on behalf of the CBTPF was the ultimate bucket list opportunity. I ran for my dear student, Maily, along with all of the children and their families affected by brain cancer. It is a memory I will carry with me for a lifetime! Thank you for the opportunity and for all you do!" — Brooke


Ryan

"This was my first time running for a charitable cause, and it was a blessed opportunity given the amount of support CBTP needs. I can't imagine what it would be like growing up with the disadvantages these children have. I put on running shoes and do a big workout, but these children have to live with health challenges every day. It's unfair. I don't know what would make it better, but it's reassuring that the folks at CBTP are dedicated to researching solutions." — Ryan (who ran on Team CBTPF with his wife, Kelly!)


Brittany

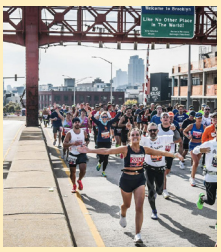
"What an awesome day for such an awesome cause! Keep up the amazing work that you do!" — Brittany


Migdalia

"Thanks for the opportunity provided to run the NYC TCS 2023 marathon on behalf of the Weill Cornell Medine brain tumor research team. It has been an honor to do so. I'm beyond thankful and proud of representing you on the event. As a mother with a special needs adult I'm very proud to be part of the community assisting the patients." — Migdalia


Gabrielle

"Running the TCS NYC for CBTPF was the most surreal experience and allowed me to be part of something so much bigger than myself. It was the most incredible day, and one that I will never forget." —Gabrielle


Ali

"I would run 26.2 miles over and over for Team CBTPF. Sunday, November 5th was a magical day – made even more special by this incredible organization." — Ali


Chris

"This is my second year running for the CBTPF. It was a tough day, but I felt so proud to have helped contribute to such a worthy cause. What could be more important than helping to provide hope to children and their families." — Chris

Sophie

"From getting to the bustling city of New York, to the anticipation built up by the expo, and to the pre-race pasta with fellow fundraisers/runners, my body was still unprepared to feel the wave of emotions as I crossed the finish line of the race. Hearing the story of Ty's dad crossing the finish line with his son in his arms really made me treasure those last few meters and take everything in. I'm so lucky to be part of such an intentional, positive cause and I feel so proud of the work that was put in this year. Thank you so much for the experience CBTP, I will not forget it!" — Sophie

Anastasia Hanafin and Ishani Ray

Welcome Anastasia and Ishani to the Children's Brain Tumor Project

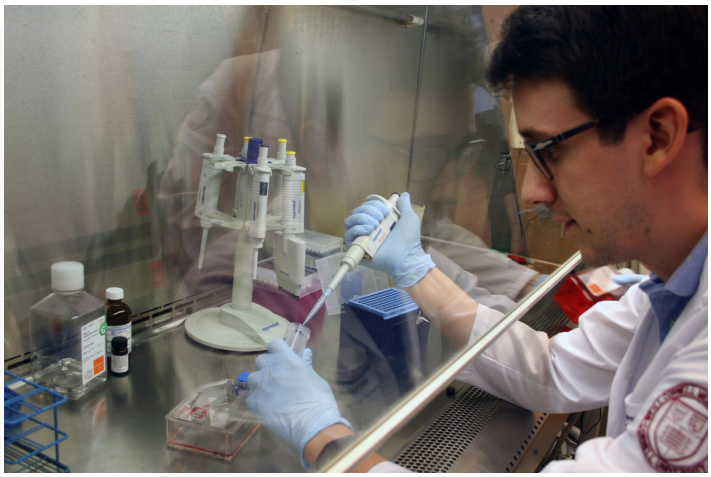
The lab team continues to grow its brain power with the hiring of two passionate research technicians, Anastasia Hanifin and Ishani Ray. Read more about why they chose to join the Children's Brain Tumor Project below.

"My name is Anastasia Hanifin and I'm a research technician at the Children's Brain Tumor Project under the mentorship of Nadia Dahmane. I graduated from Trinity College in May 2023 with a degree in Neuroscience and a minor in French. In addition to being a dancer, I was on the Varsity rowing team at Trinity. I am proud of my Ukrainian American heritage, and I am an active participant in dance and many community organizations. I am loving my time in the lab so far, and I am very grateful to be able to contribute to the amazing research that happens here. In the future, my goal is to become a doctor."

"My name is Ishani Ray and I'm a research technician in the Souweidane lab. I graduated from Oklahoma State University in May 2023 with a BS in Chemistry and Biochemistry and minors in Neuroscience, Biology, and Math. In my spare time, I enjoy pressing flowers, playing board games, and listening to TED talks. Being a part of the Souweidane lab has been an incredible learning experience, and I'm deeply grateful for the opportunities it offers for me to broaden my knowledge and learn new skills. Ideally, I'd like to pursue an MD-PhD in the future."

Addressing Disparities

The **St. Baldrick's Foundation** recently awarded the CBTP Lab with an "Infrastructure" grant to create and implement a program that addresses the disparities evident in pediatric brain tumor clinical trial enrollment. We aim to identify and address the roadblocks that prevent a diverse population from participating in clinical trials. The program is set to kick off in January, so look for news about this new team in the next newsletter.



Application Processes Underway

Rudin Fellowship and Student Summer Research Program Now Taking Applications

The 2024 application windows for both the Rudin Fellowship in Neurological Surgery and paid summer internships at the Children's Brain Tumor Project are now open.

The Rudin Fellowship

We are grateful to Jack and Susan Rudin and the Louis and Rachel Rudin Foundation for funding this fellowship, which is intended to help medical students explore a research career in pediatric neuro-oncology. Fellowship applications must be submitted by December 31, 2023. Applications will be reviewed by the Fellowship Advisory Panel with announcements being made no later than March 15, 2024.

The fellowship can be conducted any time after the student has completed a minimum of two years of medical college.

The application consists of the following:

- Scientific Proposal (Title, Objective(s), Background, Methods, and Impact: 3-page limit without references)
- Letters of reference (2)
- Sponsoring PI letter of support
- Student Affairs or Dean's letter of commitment from parent institution

Email applications to **neurosurgery-pedsfellow@med.cornell.edu.**

The Summer Research Program

Undergrad applications for a paid summer internship must be received by February 15, 2024.

Undergraduate students enrolled in U.S. universities can apply for the opportunity to experience eight weeks of hands-on experience working on research projects related to basic neuroscience and pediatric neurooncology. The competitive program requires the following:

- Student CV
- Student transcripts
- 2-3 letters of recommendation
- A personal statement indicating previous research experience and long-term goals of the student
- Interview participation for finalists

To apply for a summer position, email **cic2008@med.cornell.edu** with the required documents before February 15, 2024.

For more information on both, visit **childrensbraintumorproject.org**

"Light Up the Lab" 2023 Annual Appeal

Each year, the lab team hangs strings of lights in the hallways to honor the children who have battled brain cancer under their team's care. This heartfelt tradition serves as a poignant reminder that behind every scientific experiment, there is a child whose life could be profoundly impacted by their discoveries. The twinkling lights symbolize hope and resilience, encouraging the team to persist in their tireless efforts towards finding cures.

Before the end of the year, we will be hanging 100 tealight globes from the ceiling in the lab to literally Light Up the Lab, each of which will adorn the name of a patient treated by Dr. Mark Souweidane and/or Dr. Jeffrey Greenfield, co-founders of the Children's Brain Tumor Project Laboratory. We hope to raise $1,000 in honor of each one of those children. Some of whose inspiring stories are featured online.

We want our donors to get to know all of the children for whom we work so hard, and we invite you to visit our website throughout December as new stories are posted each week. All donors who make a year-end contribution will be provided with printable acknowledgement cards that can be given to friends and loved ones in lieu of, or in addition to, gifts. We thank you for honoring our children and supporting our progress. Visit **LightUptheLab.org.**

Children's Brain Tumor Project Wins Competitive R41 STTR Award from National Institute of Health/National Cancer Institute (NIH/NCI)

In collaboration with Dr. Neil Goldstein and Dr. Karla Frietze Miller at Skunkworx Bio and Nighthawk Biosciences, Dr. Nadia Dahmane, a principal investigator at CBTP, has recently secured an R41 STTR grant funded by the NIH/NCI. The STTR Program is designed to foster collaborative research initiatives between small businesses and research institutions, aiming to establish technical merit and feasibility for new products with market potential.

The grant will provide the necessary resources for Dahmane's lab team to further investigate atypical teratoid rhabdoid tumors (AT/RT), which are rare pediatric brain tumors notorious for their resistance to treatment. AT/RT, a lethal tumor that is predominantly found in children under the age of three, poses extreme and unique challenges; available treatments are often ineffective and extremely harsh, particularly for this young patient population.

Dr. Dahmane's team will study the use of powerful new peptide-drug conjugates (PDCs) developed by Goldstein and Frietze's team at Nighthawk Biosciences to deliver tumor-specific therapy on patient-derived preclinical models of AT/RT both *in vitro* and *in vivo*. The studies will help the team determine the effectiveness of the PDCs in reducing tumor growth, toxicity levels present in other organs during treatment, and maximum tolerated dose as a result.

This grant exemplifies how research conducted in the lab can contribute to drug development. Notably, Dr. Karla Frietze Miller was a post-doc in the CBTP lab from 2017 - 2019, before transitioning into her current position at Skunkworx Bio. Her meaningful work in our lab has translated into a sustained commitment to discovering improved continued passion for uncovering better treatment options for children with rare brain tumors, naturally fostering our collaborative efforts.

Highlights from the 2023 Cristian Rivera Foundation Celebrity Gala

Held at Capitale on November 15, the 14th Annual Cristian Rivera Foundation Celebrity Gala was a sold-out extravaganza. The star-studded event was hosted by luminaries, including Board Member and Actor Kenan Thompson, Board Member and Actor Luis Guzman, Actor Malik Yoba, and anchor of the award-winning NBC's "Today in New York", Darlene Rodriguez, a member of the foundation's Board of Directors. The room was filled with *hope*, which was the theme for the evening.

This year, the nonprofit honored Major League Baseball star Harrison Bader, CRF Executive Board Member and owner of Pulse International Realty Rena Kliot, DIPG warrior and advocate Maria Kuenster, and co-director of the Children's Brain Tumor Project at Weill Cornell Medicine Dr. Mark Souweidane.

Through donations, auction items, table sales, and more, the CRF Celebrity Gala raises funds to support the research of Dr. Mark Souweidane and Dr. Oren Becher, in addition to providing financial support to families impacted by DIPG.

In attendence were members of Dr. Souweidane's team, including (left to right) Sriram Sundaravel, Dr. Rashi Rangaraj, Dr. Rafael Uribe, Dr. Brice Martin, Dr. Rita Nguyen, Dr. Caitlin Hoffman, Dr. Mark Souweidane, and Dr. Alexandra Giantini Larsen.

Children's Brain Tumor Project
powered by families

Weill Cornell Medicine

YEAR IN REVIEW

Highlighting Lab Accomplishments from 2023

Pediatric brain tumors are very different from those found in adults, and they need to be treated differently. Funding for pediatric brain tumor research is critical, since treatments discovered for adult brain tumors are not appropriate or effective in children. Donor support of the Children's Brain Tumor Project at Weill Cornell Medicine enables the team to accelerate our progress toward uncovering cures for this devastating disease, to give these children hope, and ultimately, to keep families whole. Below are some major accomplishments as a direct result of generous donor support.

TRANSLATIONAL RESEARCH AND CLINICAL TRIALS

The Children's Brain Tumor Project is committed to building a dedicated platform for translational research. Over the past year, strong efforts have been put forth by the translational arm of our team, resulting in the following accomplishments.

- Since 2020, nonprofit support has provided critical funds to maintain the role of a designated Clinical Trials Manager. In this role, Dr. Rashi Rangaraj is responsible for protocol design and submission, regulatory approval, and funding of the CBTP therapeutic arm. Rashi's role as the CBTP clinical trials manager is focused solely on seeing clinical trials to fruition, serving as a conduit to bringing discoveries from bench to bedside while giving time back to her colleagues to continue with the research at hand.
- This past year, the first child was successfully treated on a new innovative clinical trial "Intra-Arterial Chemotherapy for Newly Diagnosed, Residual, or Recurrent Atypical Choroid Plexus Papilloma and Choroid Plexus Carcinoma Prior to Second-Look Surgery (NCT04994977)." This is the first-ever clinical trial specific to this rare diagnosis.
- As of November, we enrolled four participants in "Liquid Biopsy for Longitudinal Monitoring in Diffuse Midline Glioma Patients," an innovative clinical trial led by principal investigators Dr. Mark Souweidane of Weill Cornell Medicine, in collaboration with Dr. Alex Miller at Memorial Sloan Kettering Cancer Center and Dr. Luca Szalontay at Columbia University Irving Medical Center. The trial involves the consistent monitoring of cerebral spinal fluid (CSF) from patients with diffuse midline gliomas (DMG) and tracking the patient's response to the administration of therapeutics.
- Our precision medicine research trial, "Tissue Collection for Drug Screening and Bioanalysis (NCT04852354)" has enrolled more than 150 patients to date. 2023 highlights include the creation of new cell lines for rare pediatric brain tumors including gliomatosis cerebri (3 new cell lines), medulloblastoma (2 patient-derived xenografts), ETMR, and craniopharyngioma. This protocol allows clinicians to treat patients according to their specific tumor's molecular and cellular signatures. The goal is to enable the acquisition of tissue available for research with the prospect of enabling the development of new therapeutic avenues for patients diagnosed with cancer in the central nervous system.
- Three clinical trials are in the late stages of development at year-end, and we look forward to opening these trials for enrollment in the new year. The clinical trials will offer unique surgical approaches and drug delivery alternatives for patients with low grade gliomas, diffuse midline gliomas, and ependymoma.
- A universal Materials Transfer Agreement (MTA) between Weill Cornell Medicine and Columbia University Medical Center has been designed, approved, and implemented, allowing for seamless tissue sharing between pediatric brain tumor research labs at both institutions.

ADDRESSING DISPARITIES IN CLINICAL TRIALS

Since its inception, the CBTP has been passionate about closing the gap in healthcare disparities, specifically with regard to children with brain tumors. In 2020, the team published a peer-reviewed paper that shared statistical evidence of racial disparity in clinical trial enrollment among this patient population, and the next step is to address those findings. Thankfully, the **St. Baldrick's Foundation** shares our passion for equal access to care, and they have generously awarded us an "Infrastructure" grant to create the foundation for implementing an effective program. In 2024, our team will conduct a deep literature review to identify what the various roadblocks are to care, followed by outreach efforts to share information with a more inclusive pediatric brain tumor patient population across the tri-state area. We aim to create a pilot program that can be replicated and implemented in all densely populated areas to drastically reduce disparities in clinical trial enrollment over time.

Make a donation today at ChildrensBrainTumorProject.org

GOVERNMENT FUNDING

Research grants from the National Institutes of Health (NIH) are government-funded awards made to support a specific project representing the investigator's specific interest and competencies. These grants are very competitive, with less than 1/5 of all research applicants receiving funding (~20%).* Between 2022 and 2023, the CBTP was awarded two government grants. First is an R03 for which we initiated a collaboration with Drs K. Pollard (UCSF-Gladstone) and A. Resnick (Children's Hospital of Philadelphia) to identify genomic mutations that may disrupt the 3D genome architecture and regulate development of Atypical Teratoid Rhabdoid Tumors (ATRT). Second is an R41 grant, a more translational research program, in collaboration with Dr. Neil Goldstein (Nighthawk Biosciences, Inc), which is highlighted in greater detail in this newsletter. Government grants are a testament to the quality of the great work presented, none of which would have been possible without donor support. We strive to leverage donor funds to produce strong results that may lead to government grants.

**approximate estimate that varies annually depending on government appropriations.*

CONFERENCES

Presenting at and participating in research conferences is critical to encourage the sharing of ideas, enable collaborations, and provide attendees significant and timely updates in the field of pediatric brain tumor research. In 2023:

- Dr. Greenfield chaired the International Gliomatosis Cerebri (GC) Conference hosted at Weill Cornell Medicine, which was attended by more than 80 researchers and patient families impacted by a GC diagnosis from around the world.
- Dr. Souweidane participated in the ChadTough Scientific Retreat in Michigan, where he was paired with Dr. Sabine Mueller, pediatric neuro-oncologist at UCSF, to lead two breakout sessions on existing DIPG clinical studies and mapping out a future for additional clinical studies.
- Dr. Souweidane also participated in the DIPG/DMG symposium, an international medical research conference focusing on DIPG and DMG, which brings together 200 researchers, doctors, foundations, and families in a collaborative environment to create and fund exciting new and innovative research initiatives specific to DIPG/DMG.
- The abstract review committee selected six CBTP publications to highlight at the Society for Neuro-Oncology's (SNO) 7th Biennial Pediatric Neuro-Oncology Research Conference June 22-24, in Washington, D.C. via oral presentations and scientific posters.
- The team was proud to host the third quarterly meeting with the "Pediatric Brain Tumor Research Network (PBTRN)," a newly formed initiative sponsored by local nonprofit organizations and led by Dr. Robert Weschler-Reya, which brings together all pediatric brain tumor researchers in the NYC area. More than 60 researchers from the metropolitan area came to Weill Cornell Medicine to hear presentations from Dr. Souweidane, Dr. Greenfield, and their teams.

PEER-REVIEWED PUBLICATIONS

Peer-reviewed publications provide proof of validation for completed research because they subject the authors' scholarly work to a very high level of scrutiny from other experts in the field who evaluate whether an article is suitable for publication. The sheer volume of published work year-over-year is a testament to the hard work and dedication at the CBTP.

In 2023, members of the CBTP contributed to the bylines on 13 publications pertaining to pediatric brain tumors and served as first author (the person who contributed most to the work, including writing of the manuscript) on 5 key publications. Highlighted here are major publications from 2023, CBTP authors in bold:

- *Influence of focused ultrasound on locoregional drug delivery to the brain: Potential implications for brain tumor therapy.* **Uribe Cardenas R, Laramee M, Ray I, Dahmane N, Souweidane M, Martin B**. J Control Release. 2023 Oct;362:755-763. doi: 10.1016/j.jconrel.2023.08.060. Epub 2023 Sep 21.PMID: 37659767
- *Preclinical validation of a novel therapeutic strategy for choroid plexus carcinoma.* **Martin B, Garman T, Laramee M,** Wang A, Zhang X, Beck E, Wilson K, Klumpp-Thomas C, McKnight C, Xu X, Hagen N, Holland D, **Dahmane N**, Thomas CJ, **Souweidane M**. J Control Release. 2023 May;357:580-590. doi: 10.1016/j.jconrel.2023.04.016. Epub 2023 Apr 23.PMID: 37054779
- *Leptomeningeal dissemination in pediatric brain tumors.* **Cocito C, Martin B, Giantini-Larsen AM**, Valcarce-Aspegren M, **Souweidane MM**, Szalontay L, **Dahmane N, Greenfield JP**. Neoplasia. 2023 May;39:100898. doi: 10.1016/j.neo.2023.100898. Epub 2023 Apr 1.PMID: 37011459
- *Pediatric Postmortem Tissue Donation in the Confines of a Pandemic: A Model of Collaboration.* **Arias-Stella EU, Campbell C, Pisapia DJ, Cocito C,** McThenia S, Degliuomini M, **Greenfield JP**, Khakoo Y. Pediatr Neurol. 2023 Nov;148:138-141. doi: 10.1016/j.pediatrneurol.2023.08.009. Epub 2023 Aug 16.PMID: 37713977
- *Fifty years of DIPG: Looking at the future with hope.* **Tosi U, Souweidane M**. Childs Nerv Syst. 2023 Oct;39(10):2675-2686. doi: 10.1007/s00381-023-06037-5. Epub 2023 Jun 29