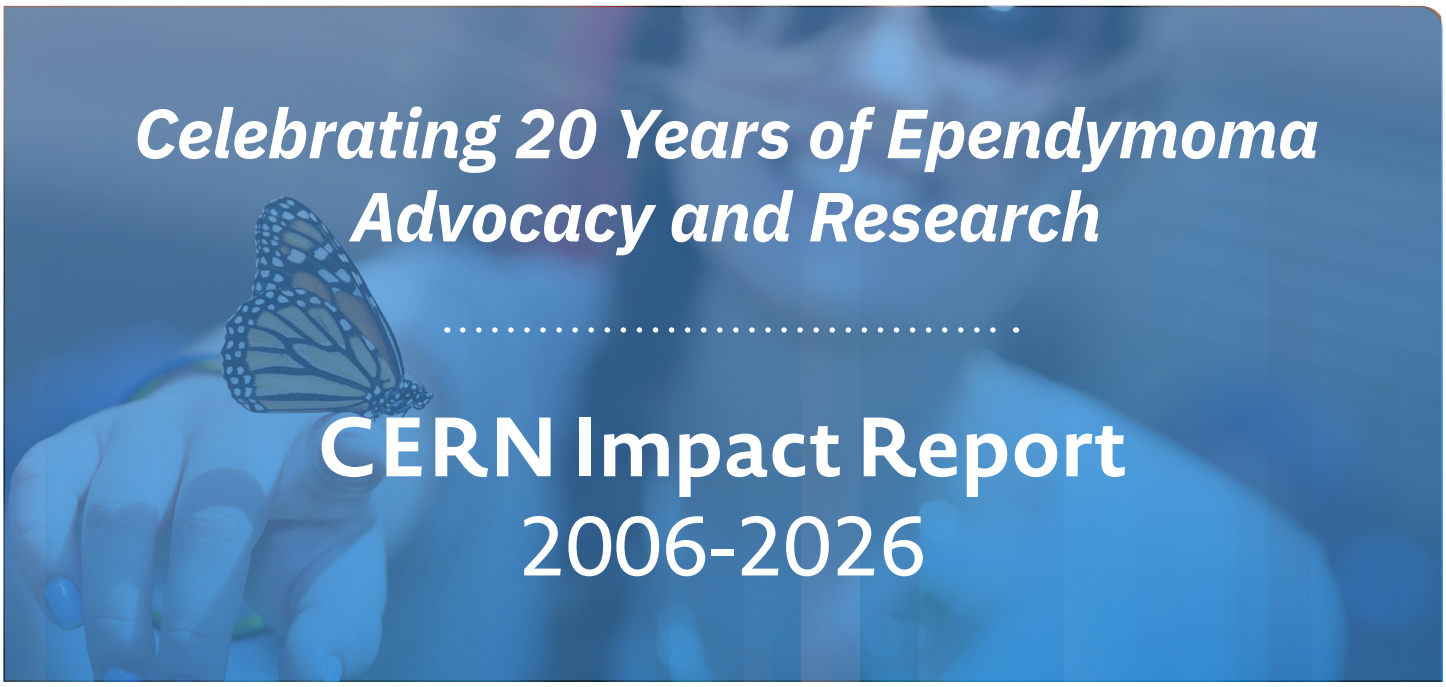
National Brain Tumor Society

cernfoundation™
Collaborative Ependymoma Reseach Network

20 YEARS OF IMPACT
2006 – 2026



# *Celebrating 20 Years of Ependymoma Advocacy and Research*

# CERN Impact Report 2006-2026

## An unwavering focus on ependymoma over 20 years.

This year marks a significant milestone as the CERN program at the National Brain Tumor Society celebrates **20 Years of Ependymoma Advocacy and Research**. Over the past two decades, our mission has been fueled by a **dedication** to patients, caregivers, and scientific breakthroughs.

Our unwavering focus on ependymoma over 20 years has shown that meaningful progress in rare diseases requires long-term commitments. Additionally, CERN carries institutional knowledge of decades of discovery and monitors research investments over time, which is paramount when resources and opportunities are limited. We recognize that each discovery builds on the last, creating cumulative impact and moving us closer to better treatments and outcomes for patients worldwide.

# Core Pillars of IMPACT

To capture the essence of this 20-year journey, the report will center on four key areas:

- **Advancing Research:** We will showcase the brilliant minds—scientists, researchers, clinicians, — whose unwavering commitment and curiosity continues to push the boundaries in ependymoma research and expose areas that need more attention through CERN's current signature awards and the distinguished fellowship program.
- **Elevating Community:** At the heart of our work are the people. We are dedicated to sharing the lived experiences of patients, families, and volunteers – ensuring their voices remain the primary catalyst for change.
- **Key Milestones and Contributions:** For the past two decades, CERN support has galvanized the field of neuro-oncology and accelerated progress in understanding the biology of the disease and identifying potential treatment strategies.
- **Honoring the Legacy:** We pause to reflect on the foundation laid by our founders and the progress made since our inception. This is a tribute to the resilience of our community and the milestones that have defined us and shape our path going forward.

# Advancing Research Through Strategic Investment

The CERN Foundation at NBTS serves as the bridge between patients, researchers, and industry — translating the nuances of the ependymoma experience into a meaningful scientific direction. We identify the most urgent unmet needs of patients and use them to guide research priorities. Something that makes our program unique is that we have a strategic plan for advancing ependymoma research, solely supported by the community through the ependymoma fund at NBTS.

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That strategy is rooted in our mission and aligns with our vision to engage, invest, and support scientific development and research efforts that influence a path towards treatments that improve the care and outcomes from people with ependymoma.

# How CERN Funds Research

Leveraging the National Brain Tumor Society's deep experience and expertise in research has increased the sophistication and management of CERN-supported grants, utilizing a combination of multi-disciplinary peer review processes and leveraging a global network of experts to identify and invite promising investigators with transformative ideas to the table. We actively manage our research portfolio to ensure projects meet clear milestones and deliver results. In addition, our granting process through CERN supported work is highly relational, leading to sustained interaction between grantee and grantor over time that produces information that drives changes beyond direct funding. In fact, most of our funded researchers have participated in CERN education and awareness activities, demonstrating their commitment beyond the funding.

**As a leader in this community, the CERN Foundation invests in targeted research efforts and develops deep-rooted relationships that yield long-term benefits for the field.**

By connecting global leaders around key topics and bringing forth the voice of the patient community, CERN changes how we learn as a continuous and relational process where insights from lived experience flow directly "upstream" to researchers. This feedback loop ensures that scientific priorities are never dictated in isolation but are constantly reshaped by the real-world needs and outcomes of the ependymoma community. Since joining the National Brain Tumor Society in 2020, CERN has supported three major signature projects and co-sponsored three fellowships totaling more than $1.3 million in dedicated ependymoma research. The following section breaks down these initiatives. *[See Appendix 1 for highlights from CERN bibliography]*

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# Signature Projects Supported by the Ependymoma Fund at NBTS

## Ependymoma Research Project Included in NBTS Flagship Research Initiative, the DNA Damage Response Consortium, 2022-2025

**Recipient:** Dr. Steve Mack, St. Jude Children's Research Hospital

**Project Title:** DNA Damage Response (DDR) Consortium - Ependymoma

**Goal:** Build a translational pipeline for ependymoma research that bridges the gap between laboratory research and clinical application

**Summary:** This initiative focuses on identifying the specific molecular drivers of tumor growth — such as the role of neurotransmitters and oncogenic fusions — to uncover new therapeutic vulnerabilities. By validating these findings in preclinical models and testing the efficacy of existing drugs like dasatinib, the grant aims to accelerate the transition from laboratory breakthroughs to clinical trials for ependymoma patients.

**Outcomes to Date:**

- Five publications in high profile medical journals, with the most prominent being Dominant clones leverage developmental epigenomic states to drive ependymoma. These findings may spark novel therapeutic strategies to test, such as forcing cells past immature developmental states to resolve the ZFTA-RELA roadblock. Nature (2026). St Jude Press Release. *[See Appendix 1 for all five publications]*
- Identification of a new therapeutic approach with the potential for translation into a clinical trial
- NBTS support leveraged to secure funding from the DOD (IDEA and IMPACT award), R01, U01 (Targeting Fusion Oncoproteins in Cancer) and a P01 (Fusion Oncoprotein Condensate Biology) federal grants totaling more than $6.4 million in follow-on funding.

**"How can you treat a disease you do not understand? The strategy of treating cells with libraries of drugs just has not worked for ependymoma. Our research across multiple papers delineates the mechanistic, developmental, and cellular basis of ependymoma, which has yielded new therapeutic insights that we can test in the laboratory." - Dr. Mack**

## By The Numbers

90% pediatric institutions/clinics, 10% adult institutions

55% basic science vs 45% translational

45% PFA EPN, 40% ST EPN, 15% SPN EPN

Initiated 9 years of ependymoma research through 3 grants

International Reach - Spanning two continents

## CERN Posterior Fossa A (PFA) Ependymoma Translational Research Award, 2024-2027

**Recipient:** Dr. Johannes Gojo, Medical University of Vienna

**Project Title:** Targeting DNA damage response to eradicate ependymoma persister cells (DEEpend)

**Goal:** Translate promising research into the clinic for a particularly aggressive type of pediatric brain tumor, Posterior Fossa A (PFA) ependymoma

**Summary:** This grant establishes a dedicated effort to tackle posterior fossa ependymoma (PFA), a particularly aggressive and underserved pediatric brain tumor. The research focuses on identifying "persister cells" — stem-like cells that survive standard treatments and cause the cancer to return. By screening existing drugs that inhibit DNA damage repair, the project aims to rapidly translate discoveries into clinical trials with new combination therapies.

## Spinal Ependymoma (SPN EPN) Award, 2025-2027

**Recipient:** Dr. Eric Holland, Fred Hutchinson Cancer Center

**Project Title:** Creating a reference landscape for spinal ependymoma

**Goal:** Define the biology of spinal ependymoma to clarify diagnosis and improve care for patients

**Summary:** This multi-year research project aims to address a critical gap in the understanding of spinal ependymoma by creating a comprehensive molecular "reference landscape." By using advanced RNA sequencing on tumor samples from around the world, researchers are working to refine how these rare tumors — particularly the mischaracterized myxopapillary subtype — are defined and classified. This international collaboration seeks to move beyond outdated diagnostic methods to ensure patients receive accurate diagnoses and more personalized care based on the specific biological drivers of their disease.

# A Decade of Advanced Research Training and Mentorship: The CERN Fellowship Program 2016-2026

By launching a fellowship program, the CERN Foundation has aimed to initiate the training of the next generation of specialized ependymoma scientists and advance patient care through innovative research. The leadership team decided the focus of the fellowship program would be on pediatric ependymoma and would be open to researchers around the world, encouraging international connection. The CERN Fellowship program quickly became a highly prestigious and sought after award.

To date, there have been a total of seven ependymoma fellowships awarded, with the most recent being initiated in 2026. Not only does the award support critical science, but it invests in a young scientist's professional development with the intention of creating more ependymoma focused programs around the world.

The momentum of the program would not have been possible without support from the key sponsor, the Robert Connor Dawes Foundation, who joined the initiative in 2018. The partnership with the RCD Foundation has expanded the reach and enhanced the durability of the program.

## Overall Outcomes

**Three fellowships alone have resulted in 38 publications and brought in nearly $7 million of follow-on funding. Drs. Pajtler, Chun, and Maaß are now leading independent research labs and have provided mentorship to other scientists in ependymoma research.**

Results from the Impact Survey are overwhelmingly positive and highlight the crucial role the CERN Fellowship program plays in the field of neuro-oncology with a high return on investment.

## 2016-2017 CERN Scientific Ependymoma Fellowship

**Vijay Ramaswamy - Mentored by Michael Taylor, M.D., PhD.**

Learn More - The Hospital for Sick Children, Toronto, Canada
Title: Personalized therapies for posterior fossa ependymoma

## 2018-2019 CERN and RCD Scientific Fellowship

**Claire King - Mentored by Richard Gilbertson, M.D., PhD.**

Learn More - University of Cambridge, UK
Title: The role of C11orf95 and C11orf95-RELA fusion in supratentorial ependymoma

## 2018-2019 CERN Scientific Ependymoma Fellowship

**Kristian Pajtler - Mentored by Stefan Pfister, M.D.**

Learn More - Hopp Children's Cancer Center Heidelberg (KiTZ), Germany
Title: Increasing diagnostic accuracy in pediatric and adult ependymoma

## 2020-2021 CERN and RCD Scientific Fellowship

**Chan Chung - Mentored by Sriram Venneti, M.D., PhD.**

Learn More - University of Michigan, Ann Arbor, MI
Title: Targeting integrated metabolic and epigenetic pathways in childhood ependymomas

## 2022-2023 CERN and RCD Scientific Fellowship

**Kendra Maass - Mentored by Kristian Pajtler, M.D.**

Learn More - Hopp Children's Cancer Center Heidelberg (KiTZ), Germany
Title: Investigating the oncogenic dependency of pediatric ependymomas on extracellular vesicle pathways and exploring their diagnostic and prognostic value

## 2024-2025 CERN and RCD Scientific Fellowship

**Samreen Shaikh - Mentored by Brandon Wainwright, PhD.**

Learn More - University of Queensland, Australia
Title: Develop and test an RNA vaccine against recurrent ependymoma tumours

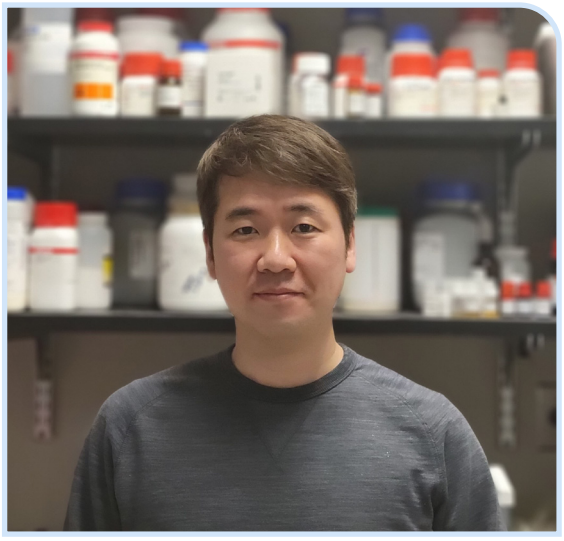
## 2026-2027 CERN and RCD Scientific Fellowship

**Siri Ippagunta - Mentored by Stephen Mack, PhD.**

Learn More - St. Jude Children's Research Hospital, Memphis, TN
Title: Targeting nuclear transport as a therapeutic vulnerability in ependymoma

# IMPACT SPOTLIGHT

## Dr. Chan Chung



CERN Foundation seed funding first introduced me to the field of ependymoma and made it possible for me to begin investigating its underlying mechanisms and potential therapeutic strategies. With this initial support, I was able to initiate research focused on the epigenetic driver, EZHIP, in PFA ependymoma and explore new therapeutic concepts that would have been difficult to pursue through conventional funding. With this foundation, I later became a faculty member and was able to start my own laboratory, where we continue to study the molecular mechanisms of ependymoma.

In my lab, we developed a quantitative reporter platform and identified candidate drugs capable of suppressing EZHIP and partially restoring repressive chromatin states linked to tumor control. Together with my students, I am expanding this work toward translational strategies aimed at improving outcomes for children with PFA ependymoma.

Receiving the CERN and RCD Scientific Fellowship was particularly meaningful to me because it was not only financial support, but also a strong vote of confidence in a high-risk idea at an early stage of my career. Unlike many traditional grants that prioritize established data and feasibility, this award recognized the potential impact of pursuing a novel therapeutic concept in a rare pediatric brain tumor.

Professionally, it gave me the momentum and credibility to build a focused research direction in ependymoma, which ultimately shaped my independent career path. Personally, it reinforced my commitment to translational research aimed at improving outcomes for children with devastating brain tumors. The patient-centered mission of the foundation made this award especially distinct and motivating compared to other funding sources I have received.

- Dr. Chung's research changed the way we understand ependymoma by identifying pharmacologic agents capable of regulating EZHIP, a key oncogenic driver of PFA ependymoma. These findings provide a potential therapeutic strategy to restore aberrant epigenetic states and open new avenues for targeted treatment approaches in PFA ependymoma.

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- Dr. Chung published two papers because of the research award with the most significant being: Targeting integrated epigenetic and metabolic pathways in lethal childhood PFA ependymomas. Sci Transl Med. 2021 Oct 6;13(614):eabc0497. doi: 10.1126/scitranslmed.abc0497. Epub 2021 Oct 6. PMID: 34613815; PMCID: PMC8762577.
- Dr. Chung's research led to the identification of a new therapeutic target and the development of a new laboratory model. Recently, a new clinical trial was launched based on work supported through the fellowship Metformin for the Treatment of Recurrent or Progressive Posterior Fossa Group A Ependymoma, PNOC041 Trial
- Data generated from the CERN and RCD Fellowship led to the Chad Tough Foundation and Michael Mosier Defeat DIPG Foundation fellowship award for $150K and helping Dr. Sriram Venneti's laboratory to win an esteemed $3 million R01 grant from the National Cancer Institute (NCI).
- Dr. Chung's career has advanced and he is now a professor and has established an ependymoma research laboratory where he mentors other junior ependymoma researchers.
- Dr. Chung is still actively researching ependymoma, with a focus on developing novel therapeutic strategies for pediatric patients.

# IMPACT SPOTLIGHT

## Dr. Kristian Pajtler

CERN funding laid the foundation for refined molecular classification and, importantly, opened a tangible path toward targeted therapies in a disease where treatment options have remained largely unchanged.

Receiving the CERN Scientific Fellowship award has been truly career-shaping for me, in a way that goes far beyond conventional project-based funding. Rather than supporting a single, well-defined study, it provided a foundation at a critical stage of my career, enabling continuity, flexibility, and the freedom to pursue emerging scientific questions as they arose. This was particularly impactful in a field like ependymoma research, where progress often depends on integrating complex datasets and following unexpected leads.

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**What stood out compared to other awards was the trust and long-term perspective associated with this support.**

It allowed me to build a coherent research program, establish collaborations, and develop models and datasets that now underpin multiple subsequent projects. In this sense, the award did not just fund experiments, it helped shape a research trajectory, strengthen my scientific independence, and position me to contribute more broadly to the field. It was not simply a grant, but a cornerstone that has had a lasting impact on my development as a clinician scientist.

- Dr. Pajtler's research discovered that supratentorial ependymoma form distinct molecular clusters driven by C11orf95 rearrangements (now designated ZFTA) beyond the canonical RELA fusion. Crucially, this work revealed that ZFTA is not merely a fusion partner but a central oncogenic driver with a shared DNA-binding domain essential for tumorigenesis.
- Dr. Pajtler's research has changed all chapters concerning ependymoma of the WHO classification, resulted in characterization of all subgroups known today, established mouse and other faithful models, and developed a new stratification scheme that will be applied in subsequent trials.
- Dr. Pajtler is now the deputy head pediatric neuro-oncology program and mentors junior ependymoma researchers. This award helped him establish an ependymoma specialized clinic at his institution.
- Since receiving the fellowship, Dr. Pajtler has authored 30 publications related to the project with the most significant because of the fellowship being: Molecular heterogeneity and CXorf67 alterations in posterior fossa group A (PFA) ependymomas. Acta Neuropathol. 2018 Aug;136(2):211-226. doi: 10.1007/s00401-018-1877-0.
- Research led to the new molecular subgrouping/diagnostic protocols, identification of a new therapeutic target, the development of a new laboratory model. In addition, Dr. Pajtler has presented this research at a major international conference, and it has been cited in WHO Blue Books and Clinical Consensus papers.
- Data generated from the CERN Fellowship led to receiving over $3M in additional funding from CRUK, DFG, and Institutional Grants. The fellowship was essential for the follow-on funding.

# IMPACT SPOTLIGHT

**Dr. Kendra Maaß**

The CERN Foundation fellowship enabled a crucial shift in my research toward developing minimally invasive approaches for monitoring pediatric brain tumors. With this support, I established workflows to analyze tumor-derived material from liquid biopsies, allowing us to detect ependymoma relapse earlier than previously possible. This marked a critical step toward translating molecular insights into clinically actionable tools.

Importantly, this work also led to the identification of novel biological vulnerabilities linked to extracellular vesicles and tumor evolution, opening new directions for targeted therapeutic strategies. The fellowship provided the flexibility to pursue high-risk, high-reward ideas that would not have been feasible otherwise, and it laid the foundation for my transition to an independent research program focused on precision diagnostics and treatment of pediatric brain tumors.

Receiving the CERN Foundation fellowship was particularly meaningful because it combined scientific support with a deep personal sense of purpose. The connection to Kim Wallgreen and Liz Dawes—who have both experienced the loss of a loved one—brought a powerful human dimension to the work and continues to motivate my commitment to improving outcomes for children with brain tumors. Unlike many traditional awards, this fellowship fostered a strong, mission-driven network that supported my growth into the pediatric brain tumor field.

- Dr.  Maaß' work has redefined disease monitoring in ependymoma by enabling early, minimally invasive detection of relapse. At the same time, she has identified previously unrecognized molecular vulnerabilities, laying the foundation for more precise, gentle and effective treatments.
- Dr. Maaß has published 6 papers because of the research award with the most significant being M-PACT leverages cell-free DNA methylomes to achieve robust classification of pediatric brain tumors Nat Cancer 7, 667–683 (2026). https://doi.org/10.1038/s43018-026-01115-4.

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- Dr. Maaß' research led to new molecular subgrouping/diagnostic protocols, the identification of a new therapeutic target, initiation of a new clinical trial, and a presentation at a major international conference.
- Dr. Maaßis now a principal investigator. The award helped her establish an independent research program on early cancer detection and liquid biopsies in children. She mentors junior scientists studying ependymoma. Additionally, she is currently leading the SIOP-Europe brain tumor group on liquid biopsies and the liquid biopsy program of BIOMECA – the Ependymoma Study of SIOPE.
- Data generated from the fellowship led to subsequent funding from U.S. and European groups totalling over €800,000.


# Elevating Community

We know that progress in rare brain tumors like ependymoma is fueled by much more than just a microscope. Even beyond the formal studies we fund, our community is constantly driving the research ecosystem forward. By recognizing that awareness and advocacy play an active role in research, we shine a light on its importance and encourage others to do the same. Additionally, we know that sharing personal narratives serve as a connection between researchers and the lived experience. NBTS and CERN play an intentional role in brain and spine tumor research through multiple activities in addition to funding including:


## Hosting Ependymoma Awareness Day

Established in 2012 by the CERN Foundation, Ependymoma Awareness Day (EAD) is a global initiative dedicated to illuminating the complexities of this rare and poorly understood disease. Spearheaded by the CERN Program at the National Brain Tumor Society, this international event is held annually. EAD serves as a vital platform to unite patients, survivors, caregivers, and researchers, amplifying the unique challenges they face.

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By increasing both public and professional recognition, we aim to accelerate the development of targeted treatments and clinical trials, striving to improve quality of life and ultimately find a cure for everyone living with ependymoma. To highlight that reach, in 2025, the CERN Facebook page alone saw 153,434 views on EAD, a major number for a technically rare and orphan disease.

On Ependymoma Awareness Day, people around the world participate in butterfly themed activities to honor loved ones with ependymoma, recognize care partners and medical workers, and to support ependymoma research efforts. The delicate and beautiful butterfly was chosen to represent the spirit of the ependymoma community as a symbol of hope through change. Just as a butterfly dramatically changes its shape, so can the meaning of hope along this journey.

### Sharing Lived Experiences

By capturing the personal stories of actual lives impacted by this disease, CERN ensures that when scientists, clinicians, industry, and government think of ependymoma, they see the faces and hear the voices behind the diagnosis. People who share their stories inspire others to share and serve as a reminder that rare does not mean alone. By providing a dedicated community for a tumor type once overshadowed by more common cancers, CERN shines a light on this poorly understood disease, propelling world class research while simultaneously building a landing place for the voices of those it serves. The importance these stories hold for other community members is immeasurable and they serve as a connecting point for people around the world. View all CERN Inspiration Stories and NBTS Ependymoma Stories.

## INSPIRATION SPOTLIGHT

### Savannah

After being diagnosed with a grade 3 anaplastic ependymoma following months of debilitating symptoms, Savannah describes the sudden and jarring transition from being a young adult focused on friends and fun to a patient undergoing surgery, radiation, and chemotherapy.

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**“It is not easy to live this way. It’s sort of strange. Since my diagnosis, I have a new sense of appreciation for life. But I also have this anger and sadness inside of me that I’m trying to work through on a daily basis. Some days I want people to pay attention to me and ask me how I’m doing, and some days I want to pretend like my cancer does not exist.” - Savannah**

Savannah highlights the invisible nature of the illness and the feeling of isolation. Ultimately, this inspiration story serves as a call for greater awareness and specialized support resources for young adults, emphasizing that the cancer experience is unique for everyone and requires a community that truly understands these specific challenges. Savannah’s video received over 35K views on YouTube

## Connecting Families to Experienced Clinicians

Acting as the connective tissue that ensures patients and caregivers have the best information available when seeking second opinions, evaluating clinical trials, and making informed decisions about their care, NBTS offers a complimentary personalized support and navigation for all brain and spine tumor families, including rare CNS tumors like ependymoma. Since joining National Brain Tumor Society in 2020, over 450 ependymoma families have received support through the PSN program with the primary reason for contact being “second opinions” followed by “clinical trials”.

“I wanted to reach out and follow up with you after we first spoke regarding my wife’s diagnosis of anaplastic ependymoma. I wanted to reach out because, at that point in time, we were truly lost and you provided literally such a lifeline in terms of information, guidance, and hope.

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We know the challenges are still present and life could change with the next MRI, but we're able to enjoy our time together and that is something I will always be grateful to CERN and NBTS for. Thank you, thank you, thank you from the bottom of my heart." - Mike Patterson, caregiver to wife, Megan, diagnosed with a grade 3 high MYCN anaplastic ependymoma

## Collaborative Education

Bringing ependymoma experts from the field of neuro-oncology directly to patients to break down complex science through free educational resources like web content, PDF guides, news articles, and videos, the CERN program strives to help families understand their diagnosis and make informed healthcare decisions. The CERN audience continues to build with over 3,750 email contacts and 5,400 Facebook followers to date. One of the most prominent resources is the Ependymoma Guide. The Ependymoma Guide, currently in its third edition, is a comprehensive resource that provides essential facts and guidance for those recently diagnosed with ependymoma. It serves as an educational toolkit for patients, caregivers, and advocates, offering detailed information on everything from treatment options and clinical trials to symptom management and support resources. More than 1,600 web users accessed the Ependymoma Guide in 2025.

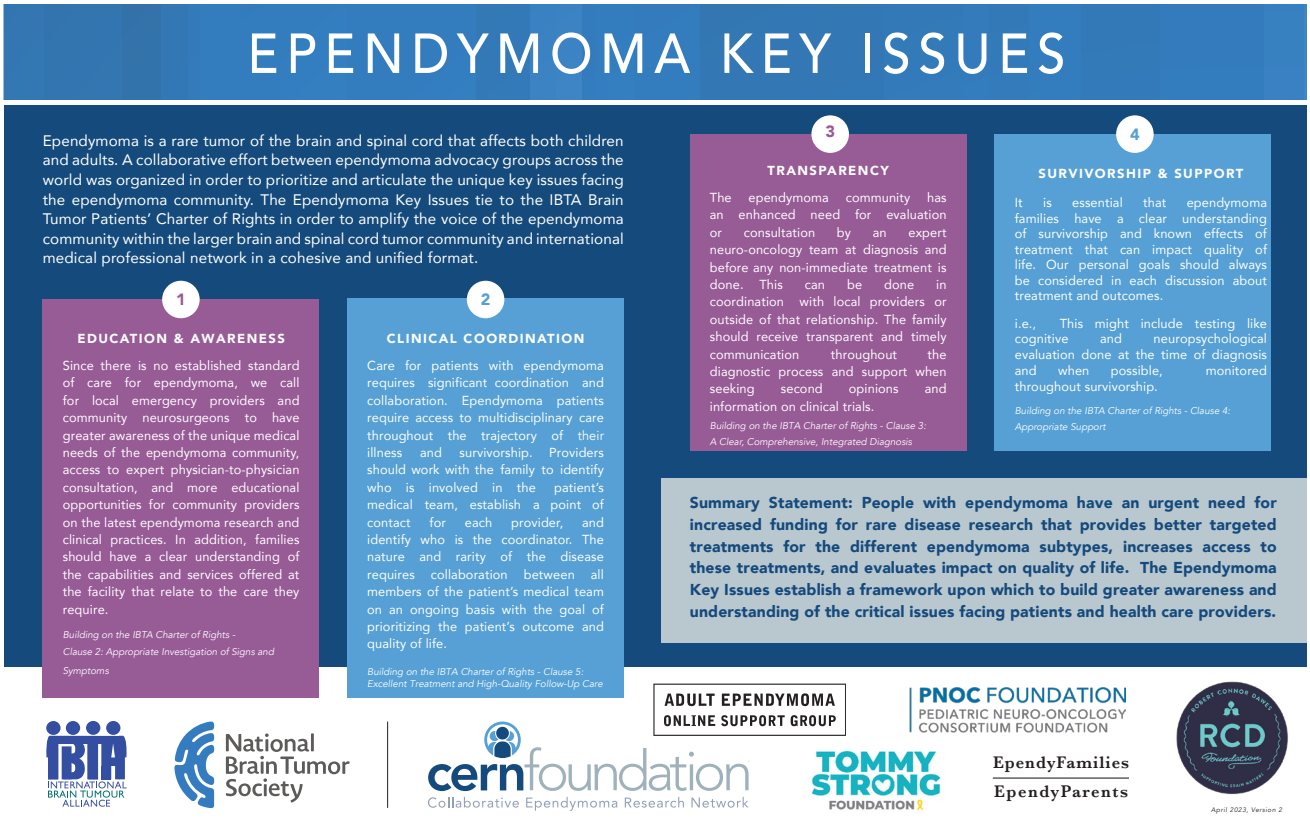
"Not only did you help me navigate my diagnosis and find doctors, but you gave me a community to belong to. I've heard so many stories that gave me hope, and I see you as a lighthouse. I once wrote that I felt lost in the ocean, and the CERN program at NBTS rescued me from that storm. Because of your organization, my life has been set on a course of progress." – Jess, Ependymoma Survivor

## Global Partnerships

The CERN program at NBTS distinguishes itself as a global leader in ependymoma advocacy and collaboration, partnering on multiple projects around the globe. A prime example is our work in identifying and presenting “Ependymoma Key Issues,” an initiative developed in tandem with the International Brain Tumor Alliance (IBTA) and other specialized advocacy groups to align global priorities (more below).

From working with the Childhood Brain Cancer Research Collaborative (CBCRC), sponsoring fellows with Robert Connor Dawes Foundation, to leading advocacy efforts in research workshops with EPN Kid in Germany, our collaborative approach ensures that global expertise is directly translated into a strategic, worldwide effort for therapeutic progress.

The Ependymoma Key Issues initiative serves as a disease-specific extension of the IBTA Brain Tumor Charter of Rights, translating broad patient protections into actionable priorities for the global ependymoma community.

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**By identifying the unique clinical and psychosocial hurdles faced by these patients, such as the need for specialized surgical expertise and long-term survivorship resources, NBTS and its international partners have created a unified framework for advocacy.**

This collaboration ensures that the aspirational rights of ependymoma patients can be seen and heard by healthcare systems and regulatory bodies worldwide.

# Key Milestones and Contributions

The Collaborative Ependymoma Research Network (CERN) was created to address a critical need to provide hope for a historically understudied disease. This effort would require that the fragmented clinical landscape be unified by bringing a multidisciplinary group of pediatric and adult neuro-oncology experts together, to provide expertise to address this area of unmet need. Over the past two decades, the CERN Foundation has achieved six transformative milestones that have reshaped patient care and scientific discovery for ependymoma by galvanizing the field.

## CERN FOUNDATION MILESTONES:

### 20 Years of Advancing Ependymoma Advocacy and Research

**2006 — Start of CERN**
CERN Foundation created to advance collaborative ependymoma research.

**2009 — Tissue Repository**
Established the tissue bank & molecular characterization initiative, launched clinical trial network for adult and ped studies.

**2010 — EPN Subtypes Recognized**
Tissue repository major contributor to finding nine EPN subtypes by 2015.

**2012 — Ependymoma Awareness**
Launched the first international Ependymoma Awareness Day.

**2016 — Fellowship Program**
Launched the CERN fellowship program to support the next generation of scientists.

**2017 — NCI-CONNECT**
Joined the NCI-CONNECT program to accelerate rare brain tumor research.

**2019 — 10 MYCN Subtype**
Identified and validated the 10th molecular subtype (MYCN amplified).

**2020 — NCCN Guidelines**
Data from CERN-0802 updated NCCN Clinical Practice Guidelines for recurrent ependymoma.

**2020 — NBTS Merge**
The CERN Foundation became a program at the National Brain Tumor Society.

**2021 — WHO Criteria Update**
Contributed to the WHO central nervous system classification update.

**2023 — NBTS DDR-C Collaboration**
Ependymoma Research included in DNA Damage Response (DDR) Consortium.

**2024 — PFA EPN Award**
Announced the inaugural CERN PFA Ependymoma Translational Research Award.

**2026 — Spinal EPN Award**
Creating a reference landscape for spinal ependymoma.

Research | Collaboration | Education | Advocacy

**The CERN Foundation is committed to improving the care and outcome of people with ependymoma through community support and research efforts.**

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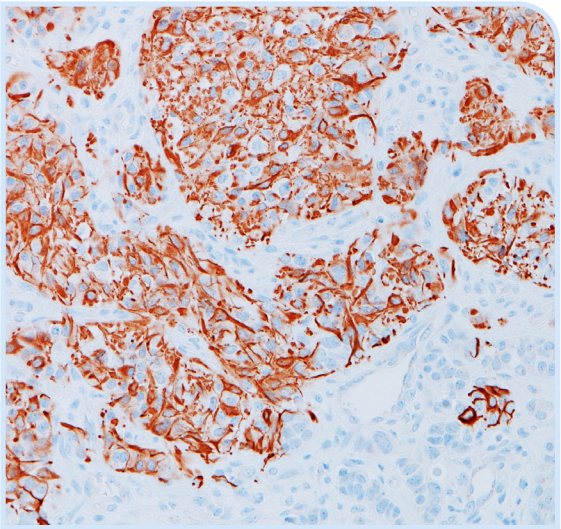
## 1. CERN Galvanized the Field and Impacted the History of Ependymoma Research

The establishment of the Collaborative Ependymoma Research Network (CERN) Foundation marked a paradigm shift in neuro-oncology by transforming ependymoma from a neglected "orphan" condition into a focal point of rigorous scientific inquiry. Central to this transformation was the foundation's commitment to consistent research support starting in 2006, which provided the catalyst for a decade of unprecedented discovery.



This first decade of research investment yielded immense scientific gains and forged deep professional connections that would anchor future projects, ultimately securing millions in follow-on funding through prestigious government, institutional, and philanthropic grants.

Prior to CERN's inception, research was fragmented and hindered by the disease's rarity; however, the foundation successfully connected the field by providing the infrastructure necessary to consolidate global expertise. This unified front led to a more sophisticated recognition of the disease, transitioning the medical community's understanding from a monolithic diagnosis to a complex landscape of molecularly distinct subgroups.

Perhaps most significantly, the CERN Foundation proved that a collaborative approach is not just viable but essential for rare disease research — triggering an increase in scientific productivity and clinical trials that has since become the gold standard for tackling rare cancers worldwide as referenced in a 2026 publication in *Neuro-Oncology*. Additionally, to expand patient access, a new nationwide network of pediatric and adult institutions was launched to conduct clinical trials specifically focused on ependymoma.

Historically, ependymoma was often grouped with other gliomas, leading to a diluted research focus. The strategic investment by CERN sparked a transition from "case report" science to high-impact genomic and clinical research.

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In the decade following 2006, the cumulative number of publications on ependymoma nearly tripled compared to the preceding decade.
This was largely driven by CERN-supported projects like the Ependymoma Outcomes Project and the Tumor Stem Cell Laboratory Models initiative. Before 2006, most papers were authored by single institutions. By 2016, the majority of high impact ependymoma research featured multi-center collaboration, a hallmark of the CERN historical model.

## Impact of CERN Foundation and Ependymoma Research Output

| Period | Est. Average of Annual EPN Publications | Research Outputs |
|---|---|---|
| Pre-CERN (2000-2005) | 170 | Fragmented reports; focus on surgery, radiation, and specific case studies. |
| Launch Phase (2006-2011) | 201 | CERN established a shift toward multi-institutional trials; molecular testing; and initial subgroup identification. |
| Growth Decade (2012-2019) | 260 | Further understanding of molecular subgroups; in vivo and in vitro high-throughput drug screens; and clinical trials. |
| Current Era (2020-2025) | 315 | WHO Classification update; targeted therapies; and immunotherapy trials. |

*Average number of annual ependymoma publications by year pulled from pubmed.gov.*

## 2. CERN Responsible for Defining Biology of Ependymoma

Over the past two decades, CERN has been responsible in recognizing that ependymoma is not a single disease, but constitutes ten subtypes with distinct genetic and molecular alterations, clinical behavior, and prognosis.

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Landmark publications by ependymoma experts including Drs. Richard Gilbertson, Kristian Pajtler, Mark Gilbert, Terri Armstrong, Ken Aldape, Eric Holland and others defined the complex biological landscape of ependymoma. CERN-affiliated research transitioned the field from vague histological grading to a sophisticated molecular framework. This evolution began with the identification of nine initial molecular subgroups (subsequently increased to 10) across the three anatomical compartments (supratentorial, posterior fossa, and spinal), providing the first rigorous evidence that location and epigenetics dictate clinical behavior.

As genomic technologies advanced, CERN's work became increasingly granular, particularly in refining the most aggressive supratentorial cases. This included the critical transition from identifying the RELA-C11orf95 fusion to the more precise definition of ZFTA-RELA fusions as the primary oncogenic driver. The network's diagnostic precision reached a new milestone with the formalization of the 10th subgroup, ST-EPN-MYCN, a distinct and highly lethal variant.

Today, the mission continues as researchers actively work to refine the spinal ependymoma classification, ensuring that the biological insights gained over the last 20 years translate into personalized, risk-adapted therapies for patients worldwide.

## 3. CERN Established the Ependymoma Tissue Study

The CERN-sponsored ependymoma tissue repository represents the largest known collection of clinically annotated adult and pediatric ependymoma tumor samples worldwide. The repository includes histology slides, tissue blocks, and extracted DNA and RNA linked to detailed clinical data from a multinational cohort. Leaders, Drs. Terri Armstrong and Liz Vera, worked at tremendous lengths to ensure proper collection, processing, and preservation of the valuable resource.

Since the repository's inception in 2009, collected data has catalyzed a paradigm shift in neuro-oncology by enabling numerous high-impact peer-reviewed publications and fostering expansive international collaborations.

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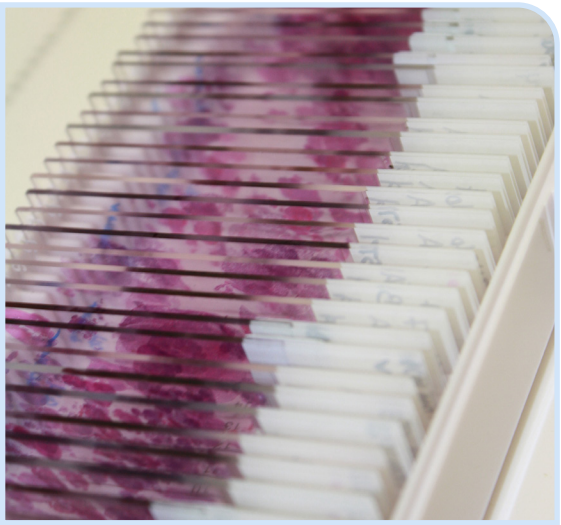

Most recently in 2025, data shared from the repository provided the essential foundation for the molecular classification of ependymoma based on anatomic location and offered the definitive evidence required for the WHO reclassification of myxopapillary ependymoma.

Furthermore, the repository has been instrumental in validating critical molecular drivers, such as the RELA fusion and MYCN-amplified subtypes, and has ensured the integration of newly defined molecular entities into official WHO classifications.

Beyond purely diagnostic advancements, the repository's insights have directly informed the design of prospective clinical trials for recurrent disease, bridging the gap between genomic discovery and improved patient management. *[See Appendix 2 to see the 14 publications that resulted from tissue and data collected through the Ependymoma Tissue Study.]*

## 4. CERN Model Led to Creation of the NCI-CONNECT Program at NCI

NCI-CONNECT (Comprehensive Oncology Network Evaluating Rare CNS Tumors) serves as a critical hub in neuro-oncology by centralizing research and clinical care for rare adult brain and spine tumors that previously lacked standardized treatment protocols. The program's importance lies in its ability to overcome the challenges of low patient numbers in rare brain and spinal tumors by fostering a collaborative, international network that connects patients, researchers, and advocacy groups to accelerate the development of precision therapies and improve survivorship.

**This multi-million dollar initiative was directly inspired by the CERN Foundation model, which proved that a dedicated, multi-disciplinary network could transform the understanding of a single rare tumor type.**

Founded by Drs. Mark Gilbert and Terri Armstrong, who created NCI-CONNECT in 2017, the CERN model of integrating laboratory science with clinical trials and patient advocacy, provided the successful blueprint for scaling this approach across 12 rare central nervous system tumors.

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Underpinned by this legacy, a major award created in part through funding from the Biden Cancer Moonshot initiative continues to expand the infrastructure needed to provide expert molecular diagnosis and innovative clinical trials, ensuring that patients with rare diagnoses are no longer left behind by traditional research models. The NCI-CONNECT: Comprehensive Oncology Network Evaluating Rare CNS Tumors, secured over $28 million in funding from federal funding from 2018-2025.

## 5. First Inclusion in NCCN Guidelines for Recurrent Ependymoma

The formal incorporation of the CERN 08-02 clinical trial into the NCCN (National Comprehensive Cancer Network) Guidelines marks a transformative milestone in neuro-oncology, establishing a much-needed evidence-based standard for adult patients with recurrent ependymoma.

Prior to this inclusion, clinicians frequently lacked robust data to guide treatment, often relying on pediatric protocols or small case series. By successfully evaluating the combination of temozolomide and lapatinib in a cohort of over 50 adult participants — an exceptional achievement for an orphan disease — CERN 08-02 provided the definitive clinical evidence required to validate this regimen as a recognized standard of care.

This advancement has had a profound impact on the community, not only by improving clinical outcomes but also by facilitating insurance authorization for these essential therapies as demonstrated in a CERN news article.

The impact of CERN 08-02 continues to resonate, shifting the treatment paradigm from empirical observation to a guideline-endorsed strategy that ensures patients receive consistent, high-quality care.

**Patients around the world can refer to this study when evaluating treatment options for recurrent ependymoma.**

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## 6. CERN Seed Grant Led to $5 Million in Federal Funding

The grant, officially titled, "Targeting the RELA-C11orf95 Fusion Oncoprotein in Pediatric Ependymoma," is a major collaborative research project funded by the National Cancer Institute (NCI) as part of the "Fusion Oncoproteins in Childhood Cancers" (FusOnC2) consortium. The grant is based on what's known as a "U54 mechanism," which requires a large, collaborative project designed to foster multidisciplinary research. This is a clear demonstration of the power of philanthropy where community dollars for the seed grant initiated a 20x multiplier, unlocking over $5 million of additional government funding.

The availability of the U54 grant, itself, is the result of the advocacy efforts of the National Brain Tumor Society and others who helped launch the Biden Cancer Moonshot in 2016. NBTS's chief executive officer, David Arons, served as a member of the Blue Ribbon Panel of experts that advised and made recommendations for the Moonshot's implementation.

One such recommendation was for an initiative to "Intensify Research on the Major Drivers of Childhood Cancers." It was through this initiative that the NIH and National Cancer Institute developed a "Fusion Oncoproteins in Childhood Cancers" consortium and made the U54 grants available.

The project was led by Richard Gilbertson at the University of Cambridge and Eric Holland at Fred Hutchinson Cancer Center from 2019-2022 and is significant because it shifts the focus toward molecularly targeted therapies that aim to be more effective and less toxic than traditional chemotherapy. The research demonstrated that the RELA-C11orf95 fusion is "sufficient" to cause these tumors on its own, making it an exceptionally high-value target for precision medicine. A notable spin off project identifies merTK as a potential therapeutic target for RELA fusion ependymoma as recently published.

Finally, the team successfully created first-of-their-kind mouse models that accurately replicate the human version of this brain tumor, allowing for more reliable drug testing before clinical trials. Achievements realized through the U54 grant have gone on to obtain further follow-on funding. This was a remarkable stride for the brain tumor community, demonstrating the importance of private philanthropy, public policy advocacy, government research funding, and team science converging to create a major opportunity for new treatment possibilities in the future. *[See Appendix 3 for a bibliography related to this project.]*

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# Honoring the Legacy

The concept for the CERN Foundation was inspired by Dallas Mathile's second recurrence of anaplastic ependymoma in 2006. During a follow-up appointment to decide on a treatment plan at MD Anderson Cancer Center, physician, Dr. Mark Gilbert, recognized a critical void in the medical landscape regarding this rare tumor. In response, Dr. Gilbert proposed a first-of-its-kind multidisciplinary consortium of adult and pediatric scientists and clinicians to pioneer a collaborative approach to the orphan disease. This critical effort was first launched through the generous support of Dallas's brother and biggest champion, Clay Mathile.

To realize this vision, Dr. Gilbert assembled a powerhouse core of experts, including renowned pediatric researcher Dr. Richard Gilbertson, pathology leader Dr. Kenneth Aldape, clinical outcomes expert Dr. Terri Armstrong, and esteemed pediatric neuro-oncologist Dr. Amar Gajjar.

This multidisciplinary team of elite laboratory and clinical researchers was chosen not only for their expertise but for their commitment to shared discovery. From 2006 to 2014, Dr. Mark Gilbert led the CERN Foundation by institutionalizing a set of core values that redefined how rare disease research is conducted. Recognizing that ependymoma suffered from scientific neglect, he established a commitment to excellence through collaboration, which became the foundation's hallmark feature.

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Gilbert maintained an environment of absolute integrity, ensuring that all efforts were guided by transparency, ethical rigor, and a singular, honest focus on the mission.

The lived experience of Dallas and Janice Mathile, and subsequently countless other community members, was instrumental in refining the program's strategic priorities, securing scientific and clinical objectives in a patient-centric framework. The community influence ensured the patient perspective remained the primary driver of all strategic decisions as was adopted as a core value early on at the start of the CERN Foundation.

## Founding CERN Foundation Centers

Children's Hospital of Chicago
Children's Hospital of Colorado
Children's Hospital of Los Angeles
Children's National Medical Center
Cincinnati Children's Hospital Medical Center
Dana-Farber and Harvard Cancer Center
Henry Ford Hospital
Huntsman Cancer Institute
University of Utah
Mayo Clinic
Memorial Sloan-Kettering Cancer Center
National Institutes of Health
Ohio State University Wexner Medical Center
St. Jude Children's Research Hospital
Stanford Children's Health
The Hospital for Sick Children (Sick Kids)
The University of Texas MD Anderson Cancer Center
University of California San Francisco
University of Miami
University of Pittsburgh Medical Center
University of Wisconsin Paul P. Carbone Comprehensive Cancer Center
Wake Forest University

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## Founding CERN Foundation Members

Kenneth Aldape
Terri Armstrong
Jennifer Atkinson
Tracy Batchelor
Eric Bouffet
Howard Colman
Lisa DeAngeles
Girish Dhall
Nicholas Foreman
Maryam Fouladi
Amar Gajjar
Elizabeth Gerstner
Mark Gilbert
Richard Gilbertson
Stewart Goldman
Kip Guy
Chas Haynes
Diane Hirakawa
Eugene Hwang
Robert Jenkins
Yasmin Khakoo
Soumen Khatua
Daniel Lachance
Glenn Lesser
Frank Lieberman
Mary Lovely
Carlos Kamiya Matsuoka
Kristin Odom
Antonio Omuro
Marta Penas-Prado
Michael Prados
Vinay Puduvalli
Roger Packer
Sonia Partap
Ian Robins
Anang Shelat
Clinton Steward
Jacqualine Stone
Tobias Walbert
Kimberly Wallgren
Khalida Wani
Jing Wu

Beginning in 2015, Kim Wallgren accepted the opportunity to lead the foundation with the goals of ensuring sustainability and long-term durability of the foundation. In close collaboration with Chas Haynes and Kristin Odom, the small but mighty team leveraged a commitment to partnership and dedication, knowing both were necessary to build on the momentum and incredible value CERN continued to offer the field. The next decade successfully expanded the foundation's reach, resulting in a marked increase in global followership, established new granting opportunities with the Fellowship Program and Signature Projects, and maximized the impact of prior research investments by ensuring critical projects were driven to completion and disseminated. By prioritizing collaboration and institutional stability, Wallgren's tenure solidified CERN's value and provided the essential groundwork for its continued impact as a cornerstone of the ependymoma community and an eligible organization for acquisition.

In 2020, the CERN Foundation officially became a designated program of the National Brain Tumor Society (NBTS), a move designed to consolidate the world's leading expertise in ependymoma with the nation's largest brain tumor advocacy platform. This strategic merger transformed years of informal collaboration into a unified partnership, combining CERN's specialized research focus with the NBTS's robust infrastructure for public policy and patient outreach. By joining forces, the organizations have streamlined the path from laboratory discovery to clinical trial, ensuring that the rare ependymoma community benefits from increased funding visibility, a broader network of support services, and a more potent, collective voice in the quest for a cure.

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NBTS's President and Chief Executive Officer, David Arons, was instrumental in shepherding a successful migration of CERN as an independent nonprofit, to becoming an integrated program of National Brain Tumor Society and continues to serve as a thought leader with program strategy. Key relationships with external advisors including Drs. Mark Gilbert, Terri Armstrong, Gajjar, Ken Aldape, Glen Lesser, Jennifer Atkinson, Gene Hwang, and Kristian Pajtler continue to be instrumental in providing guidance and scientific insight in navigating strategic decisions for the CERN program at NBTS. Through changes in organizational operation and structure, the prioritization of the patient and care partner voice has remained unchanged and continues to be the heart behind it all.

# Looking Ahead: Future Direction and Impact

By celebrating our history, we aim to inspire the next generation of community members and supporters to continue to lay the foundation for the future of ependymoma advocacy and research. There is so much work to be done to get us closer to finding new and effective treatments, so we must keep pursuing and pushing the bar higher together. This Impact Report has demonstrated the importance of disease specific focus and the critical need to protect and preserve these types of initiatives.

The call forward is simple, we need more treatment options for children and adults with ependymoma. As science advances, the CERN program at NBTS will continue to invest in best in class ependymoma research, collaborate with key leaders in the field, and provide meaningful educational resources. Additionally, having a dedicated team focus on oversight and accountability of ependymoma grants overtime, ensures the maximum value of these awards is achieved and shared with the community.

**The next two decades will bring more targeted trials, utilize emerging technologies, and build on the data infrastructure. Our mission remains: a commitment to improving the care and outcome of people with ependymoma through community support and research efforts.**

National Brain Tumor Society

cernfoundation™
Collaborative Ependymoma Reseach Network

20 YEARS OF IMPACT
2006 – 2026

# About NBTS

National Brain Tumor Society (NBTS) unrelentingly invests in, mobilizes, and unites the brain tumor community to discover a cure, deliver effective treatments, and advocate for patients and caregivers.

Building on over 30 years of experience, we are the largest patient advocacy organization in the United States committed to curing brain tumors and improving the lives of patients and families. With thousands beside us, our collective voices and actions are a powerful force for progress.

# About CERN Program

Collaborative Ependymoma Research Network (CERN) Foundation, a designated program of the National Brain Tumor Society, works to advance ependymoma research toward the development of new and better treatments for this rare brain and spinal cord tumor that impacts both adults and children. Established in 2006, CERN is committed to improving the care and outcome of people with ependymoma through community support and research efforts.

# Appendix 1

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**Molecular Classification of Ependymal Tumors across All CNS Compartments, Histopathological Grades, and Age Groups.** Pajtler KW, Witt H, Sill M, Jones DT, Hovestadt V, Kratochwil F, Wani K, Tatevossian R, Punchihewa C, Johann P, Reimand J, Warnatz HJ, Ryzhova M, Mack S, Ramaswamy V, Capper D, Schweizer L, Sieber L, Wittmann A, Huang Z, van Sluis P, Volckmann R, Koster J, Versteeg R, Fults D, Toledano H, Avigad S, Hoffman LM, Donson AM, Foreman N, Hewer E, Zitterbart K, Gilbert M, Armstrong TS, Gupta N, Allen JC, Karajannis MA, Zagzag D, Hasselblatt M, Kulozik AE, Witt O, Collins VP, von Hoff K, Rutkowski S, Pietsch T, Bader G, Yaspo ML, von Deimling A, Lichter P, Taylor MD, Gilbertson R, Ellison DW, Aldape K, Korshunov A, Kool M, Pfister SM. Cancer Cell. 2015 May 11;27(5):728-43. doi: 10.1016/j.ccell.2015.04.002. PMID: 25965575; PMCID: PMC4712639.

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**Clinical course and progression-free survival of adult intracranial and spinal ependymoma patients.** Vera-Bolanos E, Aldape K, Yuan Y, Wu J, Wani K, Necesito-Reyes MJ, Colman H, Dhall G, Lieberman FS, Metellus P, Mikkelsen T, Omuro A, Partap S, Prados M, Robins HI, Soffietti R, Wu J, Gilbert MR, Armstrong TS; CERN Foundation. Neuro Oncol. 2015 Mar;17(3):440-7. doi: 10.1093/neuonc/nou162. Epub 2014 Aug 13. PMID: 25121770; PMCID: PMC4483095.

**An in vivo screen identifies ependymoma oncogenes and tumor-suppressor genes.** Mohankumar KM, Currle DS, White E, Boulos N, Dapper J, Eden C, Nimmervoll B, Thiruvenkatam R, Connelly M, Kranenburg TA, Neale G, Olsen S, Wang YD, Finkelstein D, Wright K, Gupta K, Ellison DW, Thomas AO, Gilbertson RJ. Nat Genet. 2015 Aug;47(8):878-87. doi: 10.1038/ng.3323. Epub 2015 Jun 15. PMID: 26075792; PMCID: PMC4520751.

**C11orf95–RELA fusions drive oncogenic NF-κB signalling in ependymoma.** Parker, M., Mohankumar, K., Punchihewa, C. et al. Nature 506, 451–455 (2014). https://doi.org/10.1038/nature13109

**A prognostic gene expression signature in infratentorial ependymoma.** Wani K, Armstrong TS, Vera-Bolanos E, Raghunathan A, Ellison D, Gilbertson R, Vaillant B, Goldman S, Packer RJ, Fouladi M, Pollack I, Mikkelsen T, Prados M, Omuro A, Soffietti R, Ledoux A, Wilson C, Long L, Gilbert MR, Aldape K; Collaborative Ependymoma Research Network. Acta Neuropathol. 2012 May;123(5):727-38. doi: 10.1007/s00401-012-0941-4. Epub 2012 Feb 10. PMID: 22322993; PMCID: PMC4013829.

**An integrated in vitro and in vivo high-throughput screen identifies treatment leads for ependymoma.** Atkinson JM, Shelat AA, Carcaboso AM, Kranenburg TA, Arnold LA, Boulos N, Wright K, Johnson RA, Poppleton H, Mohankumar KM, Féau C, Phoenix T, Gibson P, Zhu L, Tong Y, Eden C, Ellison DW, Priebe W, Koul D, Yung WK, Gajjar A, Stewart CF, Guy RK, Gilbertson RJ. Cancer Cell. 2011 Sep 13;20(3):384-99. doi: 10.1016/j.ccr.2011.08.013. PMID: 21907928; PMCID: PMC3172881.

**Developing treatment strategies for rare cancers.** Atkinson JM, Gilbertson RJ. Oncotarget. 2011 Sep;2(9):657. doi: 10.18632/oncotarget.334. PMID: 21969078; PMCID: PMC3248222.

**Cross-species genomics matches driver mutations and cell compartments to model ependymoma.** Johnson RA, Wright KD, Poppleton H, Mohankumar KM, Finkelstein D, Pounds SB, Rand V, Leary SE, White E, Eden C, Hogg T, Northcott P, Mack S, Neale G, Wang YD, Coyle B, Atkinson J, DeWire M, Kranenburg TA, Gillespie Y, Allen JC, Merchant T, Boop FA, Sanford RA, Gajjar A, Ellison DW, Taylor MD, Grundy RG, Gilbertson RJ. Nature. 2010 Jul 29;466(7306):632-6. doi: 10.1038/nature09173. Epub 2010 Jul 18. PMID: 20639864; PMCID: PMC2912966.

# Appendix 2

## Publications Resulting from Ependymoma Tissue Repository [14]

**Distinct relapse pattern across molecular ependymoma types.** Obrecht-Sturm D, Schoof M, Eckhardt A, Mynarek M, Gilbert MR, Aldape K, Armstrong TS, Ramaswamy V, Bockmayr M, von Hoff K, Fleischhack G, Adolph JE, Tippelt S, Pfister SM, Pajtler K, Sturm D, Drexler R, Ricklefs FL, Stepien N, Gojo J, Pietsch T, Warmuth-Metz M, Kortmann R, Timmermann B, Haberler C, Rutkowski S, Schüller U. Neuro Oncol. 2025 Jan 12;27(1):267-276. doi: 10.1093/neuonc/noae166. PMID: 39171767

**Recurrent ACVR1 mutations in posterior fossa ependymoma.** Pratt D, Lucas CG, Selvam PP, Abdullaev Z, Ketchum C, Quezado M, Armstrong TS, Gilbert MR, Papanicolau-Sengos A, Raffeld M, Choo-Wosoba H, Chan P, Whipple N, Nasrallah M, Santi M, Ramaswamy V, Giannini C, Ritzmann TA, Grundy RG, Burford A, Jones C, Hawkins C, Venneti S, Solomon DA, Aldape K. Acta Neuropathol. 2022 Aug;144(2):373-376. doi: 10.1007/s00401-022-02435-2. Epub 2022 May 19. PMID: 35587280

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**Ultra high-risk PFA ependymoma is characterized by loss of chromosome 6q.** Baroni LV, Sundaresan L, Heled A, Coltin H, Pajtler KW, Lin T, Merchant TE, McLendon R, Faria C, Buntine M, White CL, Pfister SM, Gilbert MR, Armstrong TS, Bouffet E, Kumar S, Taylor MD, Aldape KD, Ellison DW, Gottardo NG, Kool M, Korshunov A, Hansford JR, Ramaswamy V. Neuro Oncol. 2021 Aug 2;23(8):1360-1370. doi: 10.1093/neuonc/noab034.PMID: 33580238

**High level MYCN amplification and distinct methylation signature define an aggressive subtype of spinal cord ependymoma.** Raffeld M, Abdullaev Z, Pack SD, Xi L, Nagaraj S, Briceno N, Vera E, Pittaluga S, Lopes Abath Neto O, Quezado M, Aldape K, Armstrong TS, Gilbert MR. Acta Neuropathol Commun. 2020 Jul 8;8(1):101. doi: 10.1186/s40478-020-00973-y. PMID: 32641156

**Heterogeneity within the PF-EPN-B ependymoma subgroup.** Cavalli FMG, Hübner JM, Sharma T, Luu B, Sill M, Zapotocky M, Mack SC, Witt H, Lin T, Shih DJH, Ho B, Santi M, Emery L, Hukin J, Dunham C, McLendon RE, Lipp ES, Gururangan S, Grossbach A, French P, Kros JM, van Veelen MC, Rao AAN, Giannini C, Leary S, Jung S, Faria CC, Mora J, Schüller U, Alonso MM, Chan JA, Klekner A, Chambless LB, Hwang EI, Massimino M, Eberhart CG, Karajannis MA, Lu B, Liau LM, Zollo M, Ferrucci V, Carlotti C, Tirapelli DPC, Tabori U, Bouffet E, Ryzhova M, Ellison DW, Merchant TE, Gilbert MR, Armstrong TS, Korshunov A, Pfister SM, Taylor MD, Aldape K, Pajtler KW, Kool M, Ramaswamy V. Acta Neuropathol. 2018 Aug;136(2):227-237. doi: 10.1007/s00401-018-1888-x. Epub 2018 Jul 17. PMID: 30019219

**Therapeutic Impact of Cytoreductive Surgery and Irradiation of Posterior Fossa Ependymoma in the Molecular Era: A Retrospective Multicohort Analysis.** Ramaswamy V, Hielscher T, Mack SC, Lassaletta A, Lin T, Pajtler KW, Jones DT, Luu B, Cavalli FM, Aldape K, Remke M, Mynarek M, Rutkowski S, Gururangan S, McLendon RE, Lipp ES, Dunham C, Hukin J, Eisenstat DD, Fulton D, van Landeghem FK, Santi M, van Veelen ML, Van Meir EG, Osuka S, Fan X, Muraszko KM, Tirapelli DP, Oba-Shinjo SM, Marie SK, Carlotti CG, Lee JY, Rao AA, Giannini C, Faria CC, Nunes S, Mora J, Hamilton RL, Hauser P, Jabado N, Petrecca K, Jung S, Massimi L, Zollo M, Cinalli G, Bognár L, Klekner A, Hortobágyi T, Leary S, Ermoian RP, Olson JM, Leonard JR, Gardner C, Grajkowska WA, Chambless LB, Cain J, Eberhart CG, Ahsan S, Massimino M, Giangaspero F, Buttarelli FR, Packer RJ, Emery L, Yong WH, Soto H, Liau LM, Everson R, Grossbach A, Shalaby T, Grotzer M, Karajannis MA, Zagzag D, Wheeler H, von Hoff K, Alonso MM, Tuñon T, Schüller U, Zitterbart K, Sterba J, Chan JA, Guzman M, Elbabaa SK, Colman H, Dhall G, Fisher PG, Fouladi M, Gajjar A, Goldman S, Hwang E, Kool M, Ladha H, Vera-Bolanos E, Wani K, Lieberman F, Mikkelsen T, Omuro AM, Pollack IF, Prados M, Robins HI, Soffietti R, Wu J, Metellus P, Tabori U, Bartels U, Bouffet E, Hawkins CE, Rutka JT, Dirks P, Pfister SM, Merchant TE, Gilbert MR, Armstrong TS, Korshunov A, Ellison DW, Taylor MD. J Clin Oncol. 2016 Jul 20;34(21):2468-77. doi: 10.1200/JCO.2015.65.7825. Epub 2016 Jun 6. PMID: 27269943

**Molecular Classification of Ependymal Tumors across All CNS Compartments, Histopathological Grades, and Age Groups.** Pajtler KW, Witt H, Sill M, Jones DT, Hovestadt V, Kratochwil F, Wani K, Tatevossian R, Punchihewa C, Johann P, Reimand J, Warnatz HJ, Ryzhova M, Mack S, Ramaswamy V, Capper D, Schweizer L, Sieber L, Wittmann A, Huang Z, van Sluis P, Volckmann R, Koster J, Versteeg R, Fults D, Toledano H, Avigad S, Hoffman LM, Donson AM, Foreman N, Hewer E, Zitterbart K, Gilbert M, Armstrong TS, Gupta N, Allen JC, Karajannis MA, Zagzag D, Hasselblatt M, Kulozik AE, Witt O, Collins VP, von Hoff K, Rutkowski S, Pietsch T, Bader G, Yaspo ML, von Deimling A, Lichter P, Taylor MD, Gilbertson R, Ellison DW, Aldape K, Korshunov A, Kool M, Pfister SM. Cancer Cell. 2015 May 11;27(5):728-43. doi: 10.1016/j.ccell.2015.04.002. PMID: 25965575

**C11orf95-RELA fusion present in a primary supratentorial ependymoma and recurrent sarcoma.** Cachia D, Wani K, Penas-Prado M, Olar A, McCutcheon IE, Benjamin RS, Armstrong TS, Gilbert MR, Aldape KD. Brain Tumor Pathol. 2015 Apr;32(2):105-11. doi: 10.1007/s10014-014-0205-1. Epub 2014 Nov 12.PMID: 25388523

**Clinical course and progression-free survival of adult intracranial and spinal ependymoma patients.** Vera-Bolanos E, Aldape K, Yuan Y, Wu J, Wani K, Necesito-Reyes MJ, Colman H, Dhall G, Lieberman FS, Metellus P, Mikkelsen T, Omuro A, Partap S, Prados M, Robins HI, Soffietti R, Wu J, Gilbert MR, Armstrong TS; CERN Foundation. Neuro Oncol. 2015 Mar;17(3):440-7. doi: 10.1093/neuonc/nou162. Epub 2014 Aug 13. PMID: 25121770

**Histological predictors of outcome in ependymoma are dependent on anatomic site within the central nervous system.** Raghunathan A, Wani K, Armstrong TS, Vera-Bolanos E, Fouladi M, Gilbertson R, Gajjar A, Goldman S, Lehman NL, Metellus P, Mikkelsen T, Necesito-Reyes MJ, Omuro A, Packer RJ, Partap S, Pollack IF, Prados MD, Robins HI, Soffietti R, Wu J, Miller CR, Gilbert MR, Aldape KD; Collaborative Ependymoma Research Network. Brain Pathol. 2013 Sep;23(5):584-94. doi: 10.1111/bpa.12050. Epub 2013 Mar 28. PMID: 23452038

**A prognostic gene expression signature in infratentorial ependymoma.** Wani K, Armstrong TS, Vera-Bolanos E, Raghunathan A, Ellison D, Gilbertson R, Vaillant B, Goldman S, Packer RJ, Fouladi M, Pollack I, Mikkelsen T, Prados M, Omuro A, Soffietti R, Ledoux A, Wilson C, Long L, Gilbert MR, Aldape K; Collaborative Ependymoma Research Network. Acta Neuropathol. 2012 May;123(5):727-38. doi: 10.1007/s00401-012-0941-4. Epub 2012 Feb 10. PMID: 22322993 Free PMC article.

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**The current consensus on the clinical management of intracranial ependymoma and its distinct molecular variants.** Pajtler KW, Mack SC, Ramaswamy V, Smith CA, Witt H, Smith A, Hansford JR, von Hoff K, Wright KD, Hwang E, Frappaz D, Kanemura Y, Massimino M, Faure-Conter C, Modena P, Tabori U, Warren KE, Holland EC, Ichimura K, Giangaspero F, Castel D, von Deimling A, Kool M, Dirks PB, Grundy RG, Foreman NK, Gajjar A, Korshunov A, Finlay J, Gilbertson RJ, Ellison DW, Aldape KD, Merchant TE, Bouffet E, Pfister SM, Taylor MD. Acta Neuropathol. 2017 Jan;133(1):5-12. doi: 10.1007/s00401-016-1643-0. Epub 2016 Nov 17. PMID: 27858204

**cIMPACT-NOW update 7: advancing the molecular classification of ependymal tumors**. Ellison DW, Aldape KD, Capper D, Fouladi M, Gilbert MR, Gilbertson RJ, Hawkins C, Merchant TE, Pajtler K, Venneti S, Louis DN. Brain Pathol. 2020 Sep;30(5):863-866. doi: 10.1111/bpa.12866. Epub 2020 Jun 23. PMID: 32502305

# Appendix 3

## Publications Resulting from U54 Grant [10]

**Phenotypic characterization with somatic genome editing and gene transfer reveals the diverse oncogenicity of ependymoma fusion genes.**Takadera M, Satomi K, Szulzewsky F, Cimino PJ, Holland EC, Yamamoto T, Ichimura K, Ozawa T. Acta Neuropathol Commun. 2020 Nov 23;8(1):203. doi: 10.1186/s40478-020-01080-8. PMID: 33228790

**C11orf95-RELA fusion drives aberrant gene expression through the unique epigenetic regulation for ependymoma formation.** Ozawa T, Kaneko S, Szulzewsky F, Qiao Z, Takadera M, Narita Y, Kondo T, Holland EC, Hamamoto R, Ichimura K.Acta Neuropathol Commun. 2021 Mar 8;9(1):36. doi: 10.1186/s40478-021-01135-4. PMID: 33685520

**ZFTA Translocations Constitute Ependymoma Chromatin Remodeling and Transcription Factors.** Kupp R, Ruff L, Terranova S, Nathan E, Ballereau S, Stark R, Sekhar Reddy Chilamakuri C, Hoffmann N, Wickham-Rahrmann K, Widdess M, Arabzade A, Zhao Y, Varadharajan S, Zheng T, Murugesan M, Pfister SM, Kawauchi D, Pajtler KW, Deneen B, Mack SC, Masih KE, Gryder BE, Khan J, Gilbertson RJ. Cancer Discov. 2021 Sep;11(9):2216-2229. doi: 10.1158/2159-8290.CD-20-1052. Epub 2021 Mar 19. PMID: 33741711; PMCID: PMC8918067.

**ZFTA-RELA Dictates Oncogenic Transcriptional Programs to Drive Aggressive Supratentorial Ependymoma.** Arabzade A, Zhao Y, Varadharajan S, Chen HC, Jessa S, Rivas B, Stuckert AJ, Solis M, Kardian A, Tlais D, Golbourn BJ, Stanton AJ, Chan YS, Olson C, Karlin KL, Kong K, Kupp R, Hu B, Injac SG, Ngo M, Wang PR, De León LA, Sahm F, Kawauchi D, Pfister SM, Lin CY, Hodges HC, Singh I, Westbrook TF, Chintagumpala MM, Blaney SM, Parsons DW, Pajtler KW, Agnihotri S, Gilbertson RJ, Yi J, Jabado N, Kleinman CL, Bertrand KC, Deneen B, Mack SC. Cancer Discov. 2021 Sep;11(9):2200-2215. doi: 10.1158/2159-8290.CD-20-1066. Epub 2021 Mar 19. PMID: 33741710

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**Cross-Species Genomics Reveals Oncogenic Dependencies in ZFTA/C11orf95 Fusion-Positive Supratentorial Ependymomas.** Zheng T, Ghasemi DR, Okonechnikov K, Korshunov A, Sill M, Maass KK, Benites Goncalves da Silva P, Ryzhova M, Gojo J, Stichel D, Arabzade A, Kupp R, Benzel J, Taya S, Adachi T, Shiraishi R, Gerber NU, Sturm D, Ecker J, Sievers P, Selt F, Chapman R, Haberler C, Figarella-Branger D, Reifenberger G, Fleischhack G, Rutkowski S, Donson AM, Ramaswamy V, Capper D, Ellison DW, Herold-Mende CC, Schüller U, Brandner S, Driever PH, Kros JM, Snuderl M, Milde T, Grundy RG, Hoshino M, Mack SC, Gilbertson RJ, Jones DTW, Kool M, von Deimling A, Pfister SM, Sahm F, Kawauchi D, Pajtler KW. Cancer Discov. 2021 Sep;11(9):2230-2247. doi: 10.1158/2159-8290.CD-20-0963. Epub 2021 Apr 20. PMID: 33879448

**Childhood brain tumors instruct cranial hematopoiesis and immunotolerance.** Cooper E, Posner DA, Lee CYC, Hu L, Bonner S, Taylor JT, Baldwin O, Jimenez-Guerrero R, Masih KE, Rahrmann KW, Eigenbrood J, Ngo G, Franklin VNR, D'Santos CS, Mair R, Santarius T, Craven C, Jalloh I, Moreno Vicente J, Halim TYF, Wang L, Kreigstien AR, Wainwright B, Swartling FJ, Khan J, Clatworthy MR, Gilbertson RJ. Nat Genet. 2026 Feb;58(2):317-328. doi: 10.1038/s41588-025-02499-2. Epub 2026 Feb 3. PMID: 41634415

## Follow on publications after the grant was over:

**Integrated transcriptomic landscape of medulloblastoma and ependymoma reveals novel tumor subtype-specific biology.** Arora S, Nuechterlein N, Jensen M, Glatzer G, Sievers P, Varadharajan S, Korshunov A, Sahm F, Mack SC, Taylor MD, Gujral TS, Holland EC. Neuro Oncol. 2026 Feb 1;28(2):505-519. doi: 10.1093/neuonc/noaf251. PMID: 4115938

ZFTA-RELA ependymomas make itaconate to epigenetically drive fusion expression. Natarajan SK, Lum J, Skeans JH, Nenwani M, Eyunni S, Mota M, Bayliss JM, Deogharkar A, Hamanishi ET, Pun M, Sweha SR, Hoffman S, Young E, Zhang Q, Mehta R, Animasahun O, Narayanan P, Sunil S, Parolia A, Sajjakulnukit P, Panwalkar P, Doherty R, Clausen M, Dang D, Hawes D, Yang F, Santi M, Judkins AR, Wilson Y, Vigil T, Franson A, Mortensen RM, Ozawa T, Griesinger A, Holland EC, Foreman NK, Michealraj KA, Agnihotri S, Taylor M, Gilbertson RJ, Koschmann C, Chinnaiyan AM, Lyssiotis CA, Nagrath D, Venneti S. Nature. 2026 Feb 4. doi: 10.1038/s41586-025-10005-1. Online ahead of print. PMID: 41639460

**A systems approach identifies MERTK as a therapeutic vulnerability in ZFTA-RELA-driven ependymomas.** Chan M, Zhu S, Russell ZR, Arora S, Arakaki AKS, Vaz JM, Kumasaka D, Szulzewsky F, Michealraj A, Holland EC, Gujral TS. Proc Natl Acad Sci U S A. 2026 Feb 17;123(7):e2514518123. doi: 10.1073/pnas.2514518123. Epub 2026 Feb 10. PMID: 41665993

**Childhood brain tumors instruct cranial hematopoiesis and immunotolerance.** Cooper E, Posner DA, Lee CYC, Hu L, Bonner S, Taylor JT, Baldwin O, Jimenez-Guerrero R, Masih KE, Rahrmann KW, Eigenbrood J, Ngo G, Franklin VNR, D'Santos CS, Mair R, Santarius T, Craven C, Jalloh I, Moreno Vicente J, Halim TYF, Wang L, Kreigstien AR, Wainwright B, Swartling FJ, Khan J, Clatworthy MR, Gilbertson RJ. Nat Genet. 2026 Feb;58(2):317-328. doi: 10.1038/s41588-025-02499-2. Epub 2026 Feb 3. PMID: 41634415