

# CHAMPIONS FOR CHILDREN

2024 PROGRESS REPORT



Children's Brain  
Tumor Network



# CHAMPIONS FOR CHILDREN

## OUR MISSION

The Children's Brain Tumor Network (CBTN) drives innovative discovery, pioneers new treatments, and accelerates open science to improve the health of all children diagnosed with brain tumors.

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# WELCOME

Reflecting on everything we accomplished in 2024, a powerful question arises:

## How can CBTN be Champions for Children?

Like a title-winning team, our progress in conquering pediatric brain tumors is built on collaboration, innovation, and unwavering dedication. We at CBTN are grateful to champion this vital effort.

This year, we have witnessed inspiring examples of this teamwork in action. Just as a quarterback relies on their offensive line, our researchers relied on the robust data infrastructure provided by CBTN to accelerate their discoveries. Like skilled coaches, we have seen clinicians translate groundbreaking research into improved patient care. And, like the unwavering support of fans in the stands, our patient families’ resilience and active participation have fueled our determination.

Our season of progress increased the accessibility of our comprehensive data, enabling researchers worldwide to play on the same field and accelerate their projects. The network’s collaborative research has led to significant strides in identifying personalized treatment strategies for kids who desperately need them. As with any team facing a formidable opponent, we encountered challenges. Funding remains a crucial hurdle, and the complexity of pediatric brain tumors demands diligent focus.

The 2024 CBTN Progress Report delves into the specific plays and strategies that defined our year. You will read about groundbreaking research pushing the boundaries of scientific understanding, clinical trials bringing hope to families, and our network’s unyielding commitment to every child facing a brain tumor diagnosis.

Thank you for being part of our team. Your support, dedication, and belief in our mission make our work possible. Together, we are champions for children, striving for a future in which pediatric brain tumors are no longer a threat.

With gratitude and hope,

**Executive Leadership**

Jena Lilly  
Adam Resnick  
Jay Storm

**Executive Chairs**

Sabine Mueller  
Brian Rood

**Scientific Chairs**

Carl Koschmann  
Javad Nazarian







## STATE OF PEDIATRIC RESEARCH

Pediatric cancer research is advancing rapidly through collaboration and technological innovation. Personalized medicine, powered by genomic sequencing and data-sharing initiatives like the CBTN Pediatric Brain Tumor Atlas (PBTA), is driving targeted therapies.

Immunotherapy and gene therapy offer promising new treatments, yet disparities in access and clinical trial representation remain critical challenges. Ensuring equity is essential for all children to benefit from these breakthroughs.

## A WINNING STRATEGY

CBTN is transforming pediatric brain tumor research through leading-edge technology and unprecedented collaboration.

By unifying global researchers through a centralized, cloud-based data ecosystem paired with biospecimen and preclinical models, we enable real-time analysis of vast genomic, clinical, and imaging data. This digital infrastructure accelerates discoveries and speeds translation from the lab to bedside.

Over the past year, this approach has significantly advanced breakthroughs, bringing us closer to personalized, effective treatments for children with brain tumors.



# THE M3 APPROACH

DRIVING PEDIATRIC CANCER RESEARCH FORWARD

In 2024, CBTN established a dynamic, teamwork-driven framework. This M3 approach accelerates discoveries, leading to more precise and effective treatments for children with cancer.



MULTIMODAL  
DATA  
INTEGRATION

Combining imaging, pathology, and clinical data for a comprehensive tumor profile.

MULTI-DISCIPLINARY  
COLLABORATION

Bridging expertise across cancer biology, genomics, neurosurgery, and more to tackle complex challenges.

MULTIOMIC  
ANALYSIS

Exploring genomics, proteomics, transcriptomics, metabolomics, and epigenomics to uncover tumor vulnerabilities.

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THE STATS

2024 BY THE NUMBERS

COUNTRIES REPRESENTED

9+

RESEARCH PROJECTS

350+

TOTAL ENROLLEES

7,743

SPECIMEN ALIQUOTS

78,415

CELL LINES MODELED

106

PRECLINICAL MODELS

77

PRECLINICAL MODELS

29,119

BIOSPECIMENS

9,185

PUBLICATIONS

207

PDX MODELS DEVELOPED

9

HOME FIELD ADVANTAGE

The CBTN PBTA is the world’s most extensive collection of childhood brain tumor data. This robust dataset, which contains over 5,000 unique research subjects and 40 tumor subtypes, is a key research resource.

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# MEMBER INSTITUTIONS

## ASIA

BEIJING TANTAN HOSPITAL  
NEUROSURGERY CENTER  
CHINA NATIONAL GENE BANK

## AUSTRALIA

HUDSON INSTITUTE OF  
MEDICAL RESEARCH  
SYDNEY CHILDREN'S HOSPITAL,  
RANDWICK

## EUROPE

MEYER CHILDREN'S HOSPITAL  
UNIVERSITY CHILDREN'S  
HOSPITAL, ZÜRICH

## NORTH AMERICA

AKRON CHILDREN'S HOSPITAL  
ANN & ROBERT H. LURIE  
CHILDREN'S HOSPITAL OF CHICAGO  
CHILDREN'S HEALTHCARE OF ATLANTA  
CHILDREN'S HOSPITAL OF PHILADELPHIA  
CHILDREN'S NATIONAL HOSPITAL  
CHILDREN'S OF ALABAMA  
DAYTON CHILDREN'S HOSPITAL  
DOERNBECHER CHILDREN'S HOSPITAL  
HASSENFELD CHILDREN'S HOSPITAL  
AT NYU LANGONE  
INTERMOUNTAIN PRIMARY  
CHILDREN'S HOSPITAL  
JOHNS HOPKINS ALL CHILDREN'S HOSPITAL  
JOHNS HOPKINS MEDICINE  
JOSEPH M. SANZARI CHILDREN'S HOSPITAL AT  
HACKENSACK UNIVERSITY MEDICAL CENTER  
LUCILE PACKARD CHILDREN'S HOSPITAL,  
STANFORD  
MARIA FARERI CHILDREN'S HOSPITAL  
AT WEST CHESTER MEDICAL CENTER  
MICHIGAN MEDICINE C.S. MOTT  
CHILDREN'S HOSPITAL  
NICKLAUS CHILDREN'S HOSPITAL  
ORLANDO HEALTH ARNOLD PALMER  
HOSPITAL FOR CHILDREN  
SEATTLE CHILDREN'S HOSPITAL  
ST. LOUIS CHILDREN'S HOSPITAL  
TEXAS CHILDREN'S HOSPITAL  
UC SANTA CRUZ TREEHOUSE  
CHILDHOOD CANCER INITIATIVE  
UCSF BENIOFF CHILDREN'S HOSPITAL  
UNC CHAPEL HILL —  
NORTH CAROLINA CHILDREN'S  
UNIVERSITY OF IOWA STEAD FAMILY  
CHILDREN'S HOSPITAL  
UPMC CHILDREN'S HOSPITAL OF PITTSBURGH  
WAKE FOREST BAPTIST HEALTH  
WEILL CORNELL MEDICINE

# IN LEAGUE WITH LIKE MINDS

Building a winning team takes strategy, dedication, and a shared vision. At CBTN, we see our expanding network as a championship roster, constantly strengthening to drive progress toward cures for pediatric brain tumors.

In 2024, we welcomed three new member institutions, expanding our reach to 34 contributing health systems. Together, this top team is accelerating discoveries and bringing hope to children worldwide.



## ON-SITE ALL-STARS

CBTN's clinical research coordinators are the MVPs behind the scenes, executing our research game plan with precision. These essential team members manage tumor tissue collection and data handling, ensuring every sample is carefully preserved and every patient data point is accurately recorded.

Their dedication empowers researchers and clinicians with the resources to drive breakthroughs in pediatric brain tumor treatment.

# WELCOME, NEW INSTITUTIONS



At the forefront of family-centered care, this Iowa hospital advances effective treatments for children.



Nationally recognized for pediatric excellence, this Ohio-based institution advances research and improves patient outcomes.



A leading center in Utah, this hospital's strong research focus and high patient volume make it a critical partner in the fight against pediatric brain tumors.

# POWERFUL NEW PLAYERS

Even the best teams need a strong foundation to drive progress. This year, we strengthened philanthropic support for CBTN by expanding our Executive Council and launching a new “Champion” tier—united by commitments of \$25,000 and \$10,000, respectively. These partnerships, along with the generosity of many foundations and individuals, bring vital expertise, resources, and dedication to our mission to cure pediatric brain tumors. We are deeply grateful to all who help drive impact for patients.

## OUR FIRST CHAMPION



As our inaugural CBTN Champion, Ollie’s Orchestra sets a powerful precedent. Its dedication to collaboration, data-driven research, and holistic family support embodies the spirit of our network. Integrating music therapy into its support adds a unique aspect to its mission.

## EXECUTIVE COUNCIL EXPANSION



A dedicated CBTN supporter since 2021, the foundation deepened its commitment this year by joining the CBTN Executive Council. Focused on funding medulloblastoma research, its involvement reinforces the power of collaboration to drive progress.



Founded in 2020 by a pediatric brain tumor patient, the foundation champions preclinical research on the blood-brain barrier. Its commitment provides stable funding for long-term projects, advancing critical breakthroughs in pediatric brain tumor treatment.

# EXECUTIVE COUNCIL MEMBERS



## THE POWER OF PARTNERSHIP: AMPLIFYING OUR IMPACT

By uniting expertise, resources, and networks, we accelerate research, enhance data sharing, and drive collaborative innovation for targeted treatments. Each new committed foundation also strengthens our ability to educate, advocate for, and support families navigating the challenges of a pediatric brain tumor diagnosis.





## GAME RECOGNIZES GAME

Even the most talented team recognizes the power of strategic alliances. Conquering pediatric brain tumors requires a coalition of allies, each bringing unique strengths and resources to the fight. This year, we've forged key partnerships with leading organizations, reinforcing our team and amplifying our impact.

### GOVERNMENT & RESEARCH PARTNERS

Just as a successful team benefits from the support of its league, we've partnered with influential government and research organizations:



This multi-partner collaboration with the Advanced Research Projects Agency for Health (ARPA-H) marks a major leap forward in accelerating discoveries. With a \$10 million award announced in 2024, ARPA-H supports the Center for Data-Driven Discovery in Biomedicine (D3b), operations center for CBTN, to develop the Real-time Analysis and Discovery in Integrated And Networked Technologies (RADIANT) project. RADIANT harnesses healthcare AI to revolutionize pediatric brain tumor research—saving time, improving outcomes, and setting a precedent for broader healthcare innovation beyond pediatric oncology.



Our ongoing partnership with the NIH provides crucial funding and resources for innovative infrastructure and research initiatives across the CBTN data ecosystem.

## NO "I" IN TEAM

These partnerships are not just about funding or technology; they represent a shared commitment to collaboration and innovation. Together, we are building an all-star-caliber coalition that will transform the landscape of pediatric brain tumor research.

## TECH PARTNERS HARNESSING THE POWER OF INNOVATION

In today's data-driven world, technology is a game-changer. We've partnered with leading tech companies to leverage the expertise and resources to advance science.



AWS provides the cloud infrastructure that powers the PBTA. Its scalable and secure platform enables CBTN to store, analyze, and share vast amounts of data, ensuring that researchers worldwide can access the critical information they need.



MuleSoft and Kno2 supported the initial development of RADIANT project tools, which make it easier to share and access patient data as it is logged over time. This helps support long-term patient research and create individualized care regimens for children.

### VELSERA

In 2024, partnerships drove advancements in pediatric brain tumor research. CBTN partner Velsera expanded researchers' ability to analyze vast amounts of data. With Velsera's support, researchers leverage CBTN's sophisticated cloud-based analytics tool CAVATICA to run complex data analysis pipelines and interpret large-scale genomic data.

## TRANSLATING RESEARCH INTO REAL-WORLD IMPACT

Ultimately, CBTN aims to translate research discoveries into effective treatments for children.

We've partnered with the industry innovators to accelerate this process.



This partnership enables Day One to use CBTN data and specimens to complement its clinical trial efforts. This collaboration exemplifies our commitment to closing the research and clinical application gap and enables our partners to fast-track new treatments.





# EQUIPPING OUR TEAM FOR SUCCESS

In 2024, CBTN demonstrated the power of synergy among researchers, clinicians, technology, and families. Our strategy employed research, data analysis, and patient advocacy. CBTN leveraged its vast data resources to identify promising avenues for therapeutic intervention.

# FOUNDATION FOR VICTORY

CBTN focused on key strategic priorities in 2024, which centered on attacking key aspects of research processes and data acquisition and preparation.

## AI-READY DATA

Recognizing data as the playbook for scientific breakthroughs, CBTN advanced efforts to integrate harmonized datasets with powerful platforms, ensuring AI-driven research is possible now and scalable for the future. Through initiatives like RADIANT, the network streamlined data automation, refined processes, and accelerated analysis.

## TRIAL-READY STANDARDS

We also strengthened our infrastructure, developing robust tools and pipelines to enhance data management. A key milestone was the 2024 launch of the Clinical Data Operations Core (CDOC), which will raise clinical data to trial-ready standards. This foundation sets the stage for new workflows, ensuring smooth implementation in 2025 and beyond.

## BUILDING THE ROSTER WITH DATA

The Pediatric Brain Tumor Atlas (PBTA) continued to grow through significant data-generation efforts to provide researchers with unprecedented access to comprehensive pediatric brain tumor data.

CBTN made significant strides in sequencing, notably expanding proteomic data through collaborations, including the NIH Clinical Proteomic Tumor Analysis Consortium (CPTAC). In 2024, we increased pediatric proteomic data by 300 samples (20%), maintaining the largest cohort of its kind. Plans for more predictive model sequencing in 2025 will provide an even more complete picture of these tumors.





## CBTN RESEARCH EMPOWERMENT

Our collaborative approach empowers researchers to explore novel approaches to pediatric brain tumors. This includes a comprehensive suite of initiatives to equip the CBTN team for success.

### USER SUPPORT & OUTREACH

CBTN expanded its outreach efforts to improve access to and understanding of valuable PBTA data. In 2024, CBTN trainers delivered just under 500 researcher data portal demos and course training, a 400% increase over the prior year. Through targeted workshops, webinars, and personalized consultations, CBTN empowered researchers worldwide to effectively use network resources, fostering a deeper understanding of complex datasets. This steady support and outreach ensured everyone was equipped to contribute.

### OPERATIONAL SUPPORT

Any winning organization needs strong support staff to handle logistics, and CBTN provided robust operational support to more than 350 researchers using PBTA data. This included overhauling the data and specimen request process to make managing and fulfilling requests easier. The process streamlined data access protocols, technical assistance, and computational resources, removing barriers and enabling researchers to focus on their critical work.

### NO-COST DATA ACCESS

We continued championing equitable access to pediatric brain tumor data, ensuring that financial constraints do not hinder scientific progress. This approach allowed researchers to contribute their expertise regardless of where they are based, creating a level playing field for innovation.

### EDUCATIONAL OPPORTUNITIES

CBTN offered various educational opportunities, including data analysis workshops and one-on-one live demonstrations. These initiatives empowered the next generation of researchers, ensuring a pipeline of talent dedicated to conquering pediatric brain tumors.

### IMPROVING OUTCOMES

Every biospecimen, dataset, and model directly contributes to CBTN's goal of improving the outcomes for pediatric cancer patients. This year, we saw tangible progress in translating research findings into potential clinical applications, with 21 scientific articles published in high-impact periodicals that include Nature and Cell. For example, our data sharing and collaborative analysis identified novel therapeutic targets for specific tumor subtypes, bringing us closer to personalized medicine.

*See the list of 2024 scientific publications in Appendix.*



## A SEASON TO REMEMBER

In 2024, CBTN exemplified the power of teamwork, achieving remarkable milestones through collaborative efforts. We witnessed the culmination of years of dedication, resulting in transformative projects reshaping the landscape of pediatric cancer research.

# 2024 HIGHLIGHTS

## RADIANT

Innovation by the Center for  
Data Driven Discovery in Biomedicine

The Real-time Analysis and Discovery in Integrated And Networked Technologies (RADIANT) project tackles the fragmented data landscape of pediatric cancer and rare diseases.

RADIANT underscores the power of collaboration, uniting CBTN's global network, the Pacific Neuro-Oncology Consortium (PNOC), the NIH, the Gabriella Miller Kids First Pediatric Research Program (Kids First), the INCLUDE Project (Investigating Co-occurring Conditions Across the Lifespan to Understand Down Syndrome), and technology partners Velsera and Flywheel. This dynamic coalition enhances multimodal data analysis, providing clear pathways to advance precision medicine and scale critical research for pediatric brain tumors and rare diseases.



The CBTN Pediatric Brain Tumor Atlas, (PBTA) the world's most extensive collection of childhood brain tumor data, significantly expanded in 2024. Dedicated network research coordinators worked tirelessly to ensure the meticulous collection, organization, and management of data from over 1,000 unique research subjects across 30 tumor subtypes and 800 new patient records.



Integrating the full CBTN PBTA dataset into the Gabriella Miller Kids First Data Resource Center (Kids First DRC) portal represents a major collaborative victory. Through its centralized data ecosystem, researchers worldwide now have unprecedented access to genomic, clinical, imaging, and specimen information from more than 5,800 pediatric brain tumor patients.

Data platforms, including CAVATICA and PedcBioPortal, further expand the analysis tools and resources available to PBTA users through the Kids First data ecosystem.

## CONTINUOUS IMPROVEMENT

Just as athletes strive to increase agility and reflexes, CBTN worked to stay nimble in our thinking, adopting relevant technologies to accelerate research. CBTN continued to explore leading-edge technologies, including Generative AI and Advanced Imaging, focusing on improving the detection of low-grade gliomas. Longitudinal data updates ensure that PBTA data remains current and relevant, allowing us to refine research strategies in real time.

### LEARNING FROM THE GAME

Our network's commitment to collaboration and innovation enabled CBTN to achieve remarkable progress. By embracing the "Champions for Children" philosophy, we are confident that CBTN will continue to make significant strides in the fight against pediatric brain tumors, bringing hope and healing to children and families worldwide.



CBTN recognizes that fostering collaboration and providing learning opportunities are essential to our mission of conquering pediatric brain tumors. In 2024, two pivotal events were crucial team-building exercises for our network.

ISPNO  
2024

In late June, the International Symposium on Pediatric Neuro-Oncology (ISPNO) 2024, hosted by the Children’s Hospital of Philadelphia (CHOP), transformed into a global training camp for pediatric neuro-oncology. This conference provided a comprehensive scientific program covering the latest preclinical, translational, and clinical research. During this event, CBTN **provided 69 data portal demos**, held a standing-room-only scientific session, and hosted a packed networking event, where **200 scientists, clinicians, and foundation advocates connected**.



24 CBTN  
SUMMIT  
*united minds, unlocking cures*



In October, the CBTN Summit at the AWS campus in Arlington, Virginia, served as a powerful catalyst for breakthroughs, uniting the brightest minds in science and technology. Over three days, more than 24 leading CBTN collaborators presented research progress. More than 95 researchers attended portal demos and training courses, with more than 300 attending this CBTN-hosted event. It was the largest CBTN Summit yet.

These two hallmark events provided vital learning and opportunities to connect, reinforcing the collaborative spirit that drives breakthroughs. By investing in the development of CBTN researchers and fostering collaboration, the network is building a stronger, more effective team.







## VISION FOR A BRIGHT FUTURE

As we celebrate the victories of 2024, CBTN remains steadfast in our commitment to pushing the boundaries of pediatric brain tumor research. Like any successful team, we prepare for the next year with strategic planning, advanced training, and exploring new growth options. To further accelerate progress, CBTN is developing a strategy for the future.

Our outlook centers on strengthening network infrastructure, forging strategic partnerships, and advancing precision diagnostics. We will do this while maintaining the collaborative spirit that defines CBTN.

## UNLOCKING POTENTIAL WITH AI

Developing AI-ready datasets paired with robust specimens and preclinical models will empower researchers with the necessary tools to make groundbreaking discoveries.

Progress on the RADIANT project, including developing a new pediatric brain tumor research AI and implementing CBTN use cases, will be a key driver. These developments will enhance our data analysis capabilities, allowing CBTN investigators to identify previously inaccessible patterns and insights.

## EXPANDING OUR ROSTER WITH STRATEGIC ALLIANCES

By forging strong partnerships, such as those with Day One Biopharmaceuticals, the network can accelerate the translation of research findings into clinical applications.

## POWERING INNOVATION

Advancing precision diagnostics by developing translational tools, including preclinical models and patient-monitoring technologies.

Prognosis AI and liquid biopsy initiatives will enable us to personalize treatment strategies, improve patient outcomes, and allow for more straightforward diagnosis and treatment tracking for disease progression without invasive surgeries.

## RESEARCH HAPPENS BECAUSE KIDS GIVE

In 2024, the partnership between CBTN and Gift from a Child, a foundation-funded program that supports families and clinical care teams through tissue donation facilitation, advanced new opportunities in pediatric brain tumor research. Tumor evolution brain tumor mapping helps researchers form a better understanding of brain tumors at the cellular level, which can be crucial for understanding disease progression and developing better treatments. The tumor evolution mapping process honors the legacies of children lost too soon, while accelerating discoveries that offer hope to those who may face a brain tumor diagnosis in the future.



## A RELENTLESS PURSUIT OF HOPE

Improving the long-term outlook for children receiving a brain tumor diagnosis fuels our vision for 2025. Every moment counts, and every breakthrough matters. The relentless pursuit of a cure is also driven by the love of parents, the spirit of the children affected, and the desire to prevent pediatric brain tumors from stealing a child's future. By working with our partners, we continue to be champions for children.



# APPENDIX

## 2024 CBTN LEVERAGED PUBLICATIONS

**The landscape of primary mismatch repair deficient gliomas in children, adolescents, and young adults: a multi-cohort study.** *Lancet Oncol* [Internet]. Elsevier BV; 2024 Dec 16; Available from: [http://dx.doi.org/10.1016/S1470-2045\(24\)00640-5](http://dx.doi.org/10.1016/S1470-2045(24)00640-5) PMID: 39701117

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**Automated pediatric brain tumor imaging assessment tool from CBTN: Enhancing suprasellar region inclusion and managing limited data with deep learning** [Internet]. *medRxiv*. 2024. p. 2024.07.29.24311006. Available from: <http://medrxiv.org/content/ealy/2024/07/31/2024.07.29.24311006.abstract>

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**Empowering data sharing in neuroscience: A deep learning DE-identification method for pediatric brain MRIs.** *AJNR Am J Neuroradiol* [Internet]. American Society of Neuroradiology (ASNR); 2024 Nov 12;ajnr. A8581. Available from: <http://dx.doi.org/10.3174/ajnr.A8581> PMID: 39532533

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