Research Program Guide

Vision

PBTF envisions a world where every child diagnosed with a brain tumor survives and goes on to lead a healthy, fulfilling life. This vision fuels our unwavering commitment to advancing groundbreaking science, optimizing clinical trial design, improving the safety and effectiveness of treatments, and ensuring that survivorship is defined by thriving and not merely surviving.

Why PBTF Leads

PBTF is the only national nonprofit exclusively dedicated to advancing research across all pediatric brain tumor types. Our independence from any single hospital, researcher, or tumor type allows us to serve as a trusted, neutral convener, bringing together diverse scientific disciplines, institutions, and stakeholders to accelerate discovery and translation. Our priorities are developed by the Research Advisory Committee (RAC) and informed by the Research Advisory Network (RAN) and the Technology Advisory Network (TAN), composed of leading scientists, clinicians, translational experts, computational scientists, and patient/parent advocates.

An Intentional Evolution in How We Invest

Beginning in 2026, PBTF will shift from funding individual projects to strategically building the data and analytic infrastructure that unlocks better outcomes for children. Every funded project must contribute to and draw from a shared, harmonized data ecosystem. Research is prioritized for its impact on real-time clinical decision-making. Impact is measured by clinical insights that reach children in near real time.

Proposals without a clear data strategy will not be funded.

The 2026–2030 Research Strategy

The strategy is an integrated, translation-focused framework with three connected research pillars resting on a foundational layer of infrastructure and an enabling layer of technology and data.

Infrastructure Serves as the Foundational Layer

Infrastructure is the platform on which every funded project stands. PBTF investment enables the generation, harmonization, integration, and access of pediatric brain tumor data across the broader ecosystem. Data generated through PBTF funding must be returned to the broader research community via shared resources such as NIH's database of Genotypes and Phenotypes dbGap, the Childhood Cancer Data Initiative (CCDI), the Children's Brain Tumor Network (CBTN), the Pediatric Neuro-Oncology Consortium (PNOC), and the Pediatric Cancer Data Commons (PCDC) so that every investment continues to create value for future investigators and, ultimately, future patients.

Realizing this vision requires addressing key data-sharing requirements before data reaches a shared repository. Investigators must obtain informed consent that permits broad data sharing without data use limitations (no DUL) and commit to making all data and materials available for General Research Use (GRU), allowing qualified researchers to use the data for future biomedical research. Broad consent is not simply an administrative requirement. It is what allows data to be integrated, reused, and combined with other datasets to accelerate discovery across the field.

To document that these requirements have been met, investigators must obtain an **Institutional Certification** from their institution. This certification, typically completed through the investigator's Institutional Review Board (IRB) or institutional signing official, confirms that participant consent, privacy protections, and data governance are consistent with the NIH Genomic Data Sharing Policy and support the proposed data sharing. Many institutions have already completed this certification for NIH programs such as the Gabriella Miller Kids First Pediatric Research Program. Applicants without prior experience are encouraged to work with their institutional research administration or IRB office early in the application process. This data-sharing certification is a separate form and does not substitute for the Human Subjects & Vertebrate Animals Compliance Statement required for all applicants (see Required Application Components). At the Letter of Intent (LOI) stage, applicants need only attest that their consent and institutional processes will support this requirement; the fully executed Institutional Certification itself is not required until full proposal submission or, at the latest, prior to award, since institutional review timelines can extend well beyond the LOI window.

PBTF-funded investigators are expected to incorporate these requirements into study design from the outset, ensuring that every dataset can be responsibly shared, integrated, and reused to maximize its value for children with brain tumors.

AI & Data Functions as a Horizontal Enabler

AI and data integration are not standalone components; they are embedded across all research pillars and award mechanisms. Consistent with PBTF's data-first strategy, every funded proposal must both leverage existing multi-modal datasets and contribute structured, high-quality data back to the ecosystem, addressing the longstanding fragmentation and lack of harmonization that limit progress.

Advanced technology approaches such as AI/ML, including large language models, generative AI, multi-modal analytics, and predictive modeling, are applied to enable large-scale pattern recognition, data integration across platforms, and clinically relevant insight generation across clinical, molecular, imaging, pathology, longitudinal outcomes, and real-world patient data. Transforming complex, multi-dimensional datasets into clinically actionable hypotheses and decision-support tools that can inform diagnosis, risk stratification, treatment selection, relapse prediction, survivorship planning, and therapeutic development.

Consistent with this approach, PBTF also recognizes the importance of ensuring pediatric brain tumor data are represented in the development, training, and evaluation of next-generation computational tools. By enabling responsible access to diverse, high-quality pediatric datasets, PBTF helps ensure that AI technologies are built and validated using data that reflect the unique biology and clinical characteristics of children, rather than relying solely on adult or more common disease populations.

The Three Research Pillars

Precision Medicine & Diagnosis: Development and integration of diagnostic and real-time monitoring tools, multi-modal/imaging-based biomarker discovery, and early detection strategies. Includes approaches to optimize drug delivery and overcome limitations of the blood–brain barrier, with an emphasis on generating clinically actionable, data-driven insights.

Effective Therapies: Advancing targeted and molecularly matched treatments, rational combinations and sequential regimens, and treatment optimization, including de-escalation strategies. Incorporates in silico modeling with pharmacogenomics, virtual cohorts, and data-informed therapeutic prioritization to accelerate translational impact.

Survivorship & Longitudinal Outcomes: Understanding and improving long-term outcomes through the study of late effects, neurocognitive functional studies, and quality of life. Includes relapse detection, residual disease monitoring, secondary risk predictions, and longitudinal data integration to inform lifelong, personalized care.

Data as a Force Multiplier

PBTF treats data as a strategic asset. Every investment is expected to generate, standardize, and compound value across the field. All PBTF-funded research creates multi-modal data designed for reuse beyond the original study.

Clear guardrails for data hygiene, quality, and harmonization, as established in partnership with PBTF's Technology Advisory Network (TAN) and external partners.

Every proposal must articulate how its data will be used, mined, or combined with existing assets to generate and test clinical hypotheses.

Accessing Existing Pediatric Brain Tumor Data

Applicants are expected to consider and, where appropriate, leverage existing pediatric cancer research resources before generating new data. These may include NIH-supported repositories and initiatives, collaborative research networks, and disease-specific data resources such as CBTN, PNOC, CCDI, Gabriella Miller Kids First Pediatric Research Program (Kids First), PCDC, dbGaP, and St. Jude Children's Research Hospital. Among these, CBTN provides investigators with access to integrated pediatric brain tumor research resources through the Pediatric Brain Tumor Atlas (PBTA). The PBTA combines harmonized clinical, molecular, imaging, pathology, biospecimen, and preclinical data generated through CBTN with complementary datasets from collaborative initiatives, including PNOC, and is interoperable with broader national resources such as CCDI and Kids First. Together, these interconnected resources provide investigators with a rich ecosystem for discovery, validation, and translational research while reducing duplication of efforts and maximizing the value of existing data.

The PBTA currently includes multimodal, longitudinal data from more than 6,000 patients, including clinical, genomic, imaging, digital pathology, molecular, biospecimen, and preclinical resources. Researchers can access these resources through three primary pathways:

- **Open-access data:** PedcBioPortal provides processed mutation, expression, and copy-number datasets with no login or permissions required; researchers can begin working with this data immediately.
- **Controlled-access data:** Researchers search and filter cohorts in the Kids First Data Resource Portal, then apply for access via NIH dbGaP (typically approved within two weeks). Approved data can be downloaded or analyzed directly in CAVATICA, which supports CBTN-standardized pipelines and the combination of CBTN data with a researcher's own datasets.

- **Biospecimens and preclinical models:** Researchers build a cohort in Kids First, then submit a CBTN Project Request Form for specimen or model access. The Preclinical Models Portal lists available cell lines, PDX models, and organoids.

Projects proposing to use CBTN biospecimens, preclinical models, or other network resources may undergo a feasibility assessment during the application process to help confirm the availability of requested resources and ensure the proposed work can be successfully supported. This assessment is intended to identify potential issues early and help position invited full proposals for success. Applicants relying on biospecimens, preclinical models, or datasets already in hand through prior collaborations should confirm the status of any associated Material Transfer Agreements (MTAs) or Data Transfer Agreements (DTAs) as part of the Human Subjects & Vertebrate Animals Compliance Statement.

Investigator Support: PBTF and its partners are committed to helping investigators successfully access existing resources, develop competitive applications, and prepare research-ready datasets. A variety of technical, educational, and consultation resources are available throughout the application and project lifecycle. Examples of available support, including training opportunities, office hours, data access resources, and technical assistance, are described in **Appendix A: PBTF Data Sharing & Repository Guide.**

Project Data Scenarios

Most PBTF-funded projects align with one of the following data scenarios. While each has different considerations for data generation, sharing, and integration, all are expected to contribute to a shared, interoperable pediatric brain tumor research ecosystem.

Using existing datasets such as dbGaP, PNOC, and CBTN: Investigators analyze existing clinical, molecular, imaging, or other data already available through federated resources.

Generating new data from CBTN biospecimens: Investigators receive CBTN specimens or preclinical models and generate new datasets that are incorporated into the shared research ecosystem.

Generating new data from independent cohorts: Investigators generate data from their own patients or institutional cohorts and agree to share those data in accordance with PBTF's data sharing and integration requirements.

Hybrid projects: Investigators combine data available through federated resources with independently collected cohorts or datasets to validate findings, increase statistical power, or answer broader scientific questions. These projects should clearly describe how data from each source will be harmonized, governed, and integrated while meeting all applicable consent, sharing, and repository requirements.

Although these project types have different considerations for consent, repository submission, and data integration, all PBTF-funded projects are expected to contribute to a shared, interoperable pediatric brain tumor data ecosystem. The requirements below describe the common expectations across all project types.

Data Deposit Requirements

All PBTF-funded projects must deposit research-ready data into repositories such as NIH-supported dbGaP, CBTN, PNOC, PCDC, and other secure data-sharing platforms, as appropriate for the data type, funding mechanism, and applicable data-sharing policies. Data sharing makes data available; integration makes data usable, comparable, and reusable at scale. For example, for CBTN data deposition, PBTF and CBTN will work with investigators to determine the appropriate repository strategy and support data harmonization and integration to maximize interoperability and long-term reuse across the pediatric brain tumor research ecosystem.

Repository requirements vary by data type. Individual-level human genomic data and other controlled-access datasets are typically deposited into NIH-approved repositories such as dbGaP, while harmonized clinical, molecular, imaging, biospecimen, and related research assets are integrated within CBTN, PNOC, or PCDC, depending on the originating network, to maximize discoverability, interoperability, and reuse. PBTF and CBTN will work with investigators to determine the appropriate repository strategy for each project.

Applicants are encouraged to use the technical guidance and investigator resources described in Appendix A when developing their Data Contribution & Deposit Plan.

Datasets are considered ready when they are:

- **Complete**: includes both raw and source data.
- **Interpretable:** includes sample-level context, metadata, and clear variable definitions.
- **Documented & QC'd**: includes basic quality-control metrics and a description of how the data was generated and processed.

Rule of thumb: *if another researcher could understand and reuse the dataset without contacting the originating lab, it is ready for integration.*

Detailed guidance on minimum data package requirements, repository planning, documentation, metadata, investigator resources, and modality-specific data considerations is provided in **Appendix A: PBTF Data Sharing & Repository Guide and Appendix B: Data Modality Reference.**

PBTF recognizes that preparing high-quality, reusable datasets requires dedicated expertise and resources. When additional preparation, harmonization, quality control, repository submission, or data governance activities are needed to bring data to an integration-ready state, applicants should describe these activities and associated resources **in the project budget.** If investigators lack the institutional resources or infrastructure to prepare and deposit research-ready data, PBTF may consult CBTN, as appropriate, to help define the scope of work and the associated budget required to support these activities.

Eligibility

- Principal Investigators at academic, medical, or research institutions in the U.S. and internationally.
- Career-stage requirements may vary by mechanism; early-career investigators are encouraged to apply.
- Industry partnerships are permitted and encouraged when they demonstrably accelerate translation; industry may not be the prime applicant.

- Applicants proposing to generate patient-derived data must confirm at the Letter of Intent (LOI) stage that existing or planned participant consent supports broad data sharing through General Research Use (GRU) without data use limitations (DULs) that would prevent data sharing and integration. At the LOI stage, this confirmation may be provided as a written attestation; the fully executed Institutional Certification is not required until full proposal submission or, at the latest, prior to award. Projects that cannot meet these requirements will not advance to full proposal review.
- All applicants must commit to the data sharing, deposition, and integration requirements described in this guide.
- PBTF maintains a tumor-, institution-, and researcher-agnostic posture: prior PBTF funding neither advantages nor disadvantages an application.

Required Application Components

These components are required of every application across all award levels. Mechanism-specific page limits and additional requirements are defined in the Mechanism documents.

- **Translational pathway statement**: How the work moves from discovery toward clinical impact within the funding term.
- **Data contribution & deposit plan**: Data types to be generated, repository strategy (e.g., CBTN, dbGaP, or other appropriate repositories), how the data will meet individual database integration standards where applicable, confirmation that consent supports General Research Use (GRU) without Data Use Limitations, and the proposed timeline for data deposition, sharing, and integration.
- **Research resource & data use strategy**: Existing relevant data resources the project will leverage and how, including the rationale for generating new data where appropriate.
- **Technology (AI/ML) integration plan**: Methods (including de novo models), validation strategy where applicable, and how the approach scales across federated, multi-modal datasets.
- **Human Subjects & Vertebrate Animals Compliance Statement**: Current IRB approval/exemption status for the proposed human-subjects research protocol (distinct from the Institutional Certification for data sharing described above), and IACUC approval status for any vertebrate animal work, including PDX and organoid model generation. Applicants may state "Not Applicable" if neither human subjects nor vertebrate animals are involved in the proposed work. For international sites, the equivalent institutional ethics or research-ethics board approval applies.
- **Team & Equity Statement**: A brief statement addressing team credibility, confidence in completing the proposed timeline, and how the work's findings, tools, or resources will reach across tumor types and historically underserved populations, where appropriate.
- **Risk articulation**: A 1-paragraph statement identifying the highest-risk assumption in the proposed work and what a negative result would teach the field (**only for Idea award**).
- **Bridge to next funding**: A short plan describing how a successful outcome positions the work for the next PBTF award mechanism, external follow-on funding (e.g., an NIH R01), or an industry partnership.

Guidance for preparing the Data Usage & Mining Strategy, Data Contribution & Deposit Plan, and Technology (AI/ML) Integration Approach, including recommended content for the LOI and Full Proposal, is provided in Appendix A.

Review Process & Annual Cycle

The PBTF grant cycle runs from August through June. From strategy development to the award announcement, the process typically spans approximately 9 months, though timelines can be accelerated when opportunities, funding, and leadership alignment enable faster progress.

Date	Application Milestone
Aug. 18, 2026	Topic-Specific Calls released; Letters of Intent (LOIs) open
Sept. 18, 2026	LOIs are due
Nov. 1, 2026	Applicants invited to submit a full proposal are notified
Jan. 12, 2027	Full proposals are due
Jan.-Mar. 2027	Scientific peer review. Applicants may be contacted to address reviewer questions or provide additional information
Apr. 26, 2027	Funding decisions communicated to applicants; award notifications issued
May 30, 2027	Award agreements finalized and recipients publicly announced
Jun. 1, 2027	Funding begins

Review Criteria

All proposals are scored against five criteria. Mechanism-specific weightings are defined in the Mechanism One-Pagers.

- **Scientific Significance**: Importance of the question; potential to alter the trajectory for children with brain tumors.
- **Rigor & Translational Pathway**: Clarity and credibility of the path from this work to future clinical impact, improved patient outcomes, or changes in clinical practice.
- **Data Strategy & Harmonization**: Quality of the data generation, deposit, integration, and reuse plan, including alignment with PBTF's data-sharing requirements and, where appropriate, data integration standards.
- **Technology (AI/ML) Integration**: Rigor of the AI/ML approach and validation strategy, including how the approach scales across federated, multi-modal datasets.
- **Team, Governance & Equity**: Team credibility, timeline confidence, reach across tumor types and underserved populations

PBTF Research Core Values

Our research values ensure that bold science is advancing collaboratively; discoveries are shared openly, and progress reaches every child equitably and urgently.

Innovation: Bold, high-risk/high-reward research that can transform the pediatric brain tumor landscape.

Collaboration: Cross-sector, multi-institutional, and international partnerships that break down silos.

Equity: Every child benefits from advances in pediatric brain tumor research, regardless of location, background, diagnosis, or socioeconomic status.

Patient-Centeredness: Research priorities, designs, and outcomes are shaped by the lived experiences of children and families.

Accountability: Clear benchmarks; every investment is expected to contribute meaningfully to improved outcomes for children with brain tumors through discovery, translation, or clinical advancement.

Transparency: Outcomes shared openly; decisions communicated clearly.

Urgency: Time is critical for children facing brain tumors; we prioritize what accelerates discovery and delivery.

For Mechanism rules, see the Idea / Impact / Innovation one-pagers. For current scientific priorities, see the Annual Topic Specific Calls.