Factors and Forces in Neuro-Oncology Therapy Development: Planning for Wins by 2030

July 17, 2025

On July 17, 2025, the National Brain Tumor Society (NBTS) convened a multi-stakeholder, multi-disciplinary group of approximately 55 distinguished brain tumor experts, industry leaders, regulatory officials from the U.S. Food and Drug Administration (FDA), and patient and care partner representatives for an all-day Research Roundtable meeting. The goal of this Roundtable was to take stock of neuro-oncology product development at the midpoint of the decade and identify realistic, high-impact strategies to leverage progress and reduce barriers to therapy development over the next five years and beyond. Rather than rehashing well-known challenges such as chronic underfunding and translational bottlenecks, the meeting was designed to surface actionable, forward-looking priorities that can be pursued within current resource constraints. Participants engaged in a workshop-style review of the state of the field, followed by small-group discussions to prioritize topics for future work to help overcome barriers to brain tumor treatment development and turn brain tumors into a manageable, and ultimately survivable, disease by 2030.

State of the Field at the Mid-Decade Point

The first half of the decade brought meaningful structural change to neuro-oncology drug development. The COVID-19 pandemic, while disruptive, served as a catalyst that normalized telemedicine, expanded interest and viability of decentralized trial models, accelerated regulatory-industry collaboration, and highlighted the credibility real-world evidence. Scientific and technological progress — including artificial intelligence (AI) in drug discovery, next-generation sequencing and comprehensive biomarketing testing, advanced imaging, and liquid biopsies — have drastically improved the landscape of diagnosis and treatment monitoring, as well as opened the possibility in some cases for precision targeting with newly approved agents. At the same time, these developments were bolstered by the World Health Organization's 2021 molecular reclassification of brain tumors, which has further transformed patient management and clinical trial design. Additionally, immunotherapy has matured with new targets and combination approaches, and emerging interventions such as focused ultrasound and sonodynamic therapy are entering the pipeline. Finally, the growth of active partnerships with patient advocates has advanced critical research in quality of life (QoL) metrics, legislative funding panels, data initiatives, and public awareness campaigns.

Even so, the investment climate has been volatile, accelerated approval pathways face heightened scrutiny, and incremental survival gains have placed greater emphasis on survivorship, neurocognitive outcomes, and quality of life alongside traditional efficacy endpoints.

Key Themes and Priorities for Progress

Across plenary and breakout discussions, participants identified a set of priority areas for the field to focus on through 2030 to transform brain tumors into a manageable, and ultimately survivable, disease:

- **Expand partnerships.** Addressing barriers to treatment development will require deeper collaboration among trial networks, cooperative groups, community and rural oncology sites, foundations, and advocacy organizations.

- **Strengthen data, AI, and research infrastructure.** Integrating molecular, imaging, and clinical data — and applying AI to large-scale datasets — can deepen understanding of tumor heterogeneity and improve clinical decision support. Importantly, the field should learn from every dataset, including trials that miss their primary endpoints, through retrospective tissue correlates and subpopulation analyses.

- **Advance trial innovation and access.** Pragmatic and decentralized trial designs, supported by FDA interest and guidance, can extend clinical trial availability into community and rural settings. Neoadjuvant and window-of-opportunity models may offer alternatives to designs that require patients to be randomized into standard-of-care arms.

- **Minimize patient burden and emphasize quality of life.** Continued validation of less invasive tools such as liquid biopsy may reduce reliance on more invasive procedures, and trials should consistently include endpoints that capture how patients feel and function, not only how long they live.

- **Create standardization and quality control.** National and international protocols for biospecimen collection, preservation, and chain of custody — along with strategies to maximize the value of every tissue sample — would improve comparability and research value across institutions.

- **Promote strategic funding, advocacy, and policy engagement.** In an era of constrained federal funding, a coordinated prioritization framework could help guide philanthropic, government, and venture capital investment. Documented, patient-centered stories of the impact of funding gaps are one way to inform policy, and continued constructive engagement with the National Institutes of Health (NIH) remains essential.

Participants also affirmed practices worth continuing — taking "shots on goal," investing in strong preclinical modeling, and building relationships between research and clinical experts — while calling for the field to move faster, avoid running trials without tissue collection and analysis, and embrace a

culture shift that treats informative negative trials as valuable learning experiences rather than failures.

The Patient as Partner

A consistent throughline of the meeting was the evolving role of patients and care partners — from occasional consultation to active partnership in shaping research design, drug development, and clinical decision-making. Participants emphasized the continued importance of access to clear, accessible information and educational resources, from explaining molecular classifications to helping newly diagnosed patients understand their options before beginning standard of care.

Path Forward

NBTS will work with the Research Roundtable Steering Committee to identify topics for upcoming Roundtable sessions and to advance working groups and collaborative activities in the priority areas surfaced by participants. Potential next steps discussed at the meeting include:

- Developing centralized, equitably accessible tissue repositories;
- Piloting pragmatic trial elements within existing cooperative groups and platforms;
- Advancing multi-center liquid biopsy validation;
- Building a compelling investment narrative for venture capital and philanthropic partners; and
- Preparing actionable proposals to inform research funding priorities.

Throughout, participants pointed to NBTS's role as an impartial convener — using position papers, consensus statements, and convenings to provide a unified voice for the field and to keep the focus on the most feasible, high-impact steps toward making brain tumors manageable, and ultimately survivable, by 2030.