



Brain Tumor Quality of Life

RESEARCH AGENDA

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

Learn more about the Quality of Life Research Initiative and all of our programs and services by visiting

[BrainTumor.org](https://www.brainumor.org)



Brain Tumor Quality of Life (QoL) Research Agenda

Executive Summary

We need breakthroughs - now. Patients with central nervous system (CNS) tumors and their loved ones are waiting for better treatments, a better quality of life (QoL), and cures. Patients, caregivers or care partners, researchers, government officials, and health care providers must work together to disrupt the status quo and invest in promising treatments and technology to achieve life-saving outcomes.

To achieve breakthroughs, we must bring together the best of research and patient services to create comprehensive resources for patients, families, care partners, researchers, government officials, and medical professionals. By investing strategically and wisely in innovative research, National Brain Tumor Society (NBTS) is working to advance treatments, find a cure for brain tumors, and improve QoL for people living with brain tumors and their loved ones.

In an effort to expand brain tumor research and specifically focus on QoL issues, NBTS initiated the QoL Institute project through funding from the Patient-Centered Outcomes Research Institute® (PCORI®) in 2021. Over the past two years, NBTS engaged in an in-depth stakeholder community engagement process to discuss key issues, surface previously under-recognized concerns, and develop a brain tumor-specific research agenda to serve as a resource for others working in the field, both nationally and internationally.

Background

More than 1 million people in the United States live with a primary brain tumor¹. The level of severity of these tumors results in a diverse spectrum of prognoses, experiences, and related QoL needs. For those living with a brain tumor, QoL may encompass a wide range of physical, psychosocial, and emotional concerns stemming from loss of functionality and changes in personality. Simultaneously, care partners experience their own QoL needs as they accompany their loved ones through the brain tumor experience.

While both survival and QoL reside as a top priority for most patients and care partners, research funding is more abundant in the domains of biological discovery and treatment.

¹ *CBTRUS Statistical Report: Primary Brain and Other Central Nervous System Tumors Diagnosed in the United States in 2016–2020*

Process to Increase Patient-Centered Brain Tumor QoL Research

To address the lack of funding and the need for inclusion of patient-centered QoL research in brain tumors, NBTS set out to:

- Identify important QoL research topics and questions.
- Advance QoL research for people facing brain tumors.
- Foster a community of like-minded stakeholders to enrich the field of QoL related to central nervous system (CNS) tumors in adults and children.
- Improve patient-centered and comparative effectiveness research and clinical practice as it pertains to brain tumor QoL.
- Encourage appropriate patient and community partnerships to support future QoL research using innovative approaches for patient-partner engagement.
- Amplify the importance of QoL research in the brain tumor space.

FIGURE 1



QoL Project Activities

In 2021, NBTS was awarded a Patient-Centered Outcomes Research Institute® (PCORI®) Eugene Washington PCORI Engagement Award (EACB-23261) to develop a brain tumor-specific QoL research agenda.

Over the last two years, NBTS has increased its capacity to support QoL research specific to brain tumors. As part of [this initiative](#), NBTS collaborated with patients, care partners, researchers, clinicians, pharmaceutical companies, and others as part of a stakeholder advisory group to establish a comprehensive research agenda (Figure 1).

The stakeholder advisory group members are part of NBTS's community networks and collaborators from the Brain Cancer Quality of Life Collaborative (BCQoLC). The BCQoLC was established in 2017 with a [PCORI Pipeline to Proposal Award](#) to engage brain tumor stakeholders in establishing research priorities in palliative care for adults with brain cancer. This NBTS QoL project builds upon previous NBTS and BCQoLC efforts and was led by NBTS and two leading brain tumor QoL researchers who actively participated in the initiative.

Key activities of this project included:

- Creating a definition of QoL specific to the brain tumor community
- Forming a stakeholder advisory group with at least half of all members identifying as patients or care partners
- Hosting two in-person roundtable meetings with a broader range of brain tumor community stakeholders, one for adults and the other for pediatric, adolescent, and young adult (AYA) populations
- Establishing a patient-centered brain tumor QoL research agenda

To start, advisors reviewed the QoL landscape and existing research and tools to identify opportunities to leverage and collaborate.

Brain Tumor QoL Definition and Components

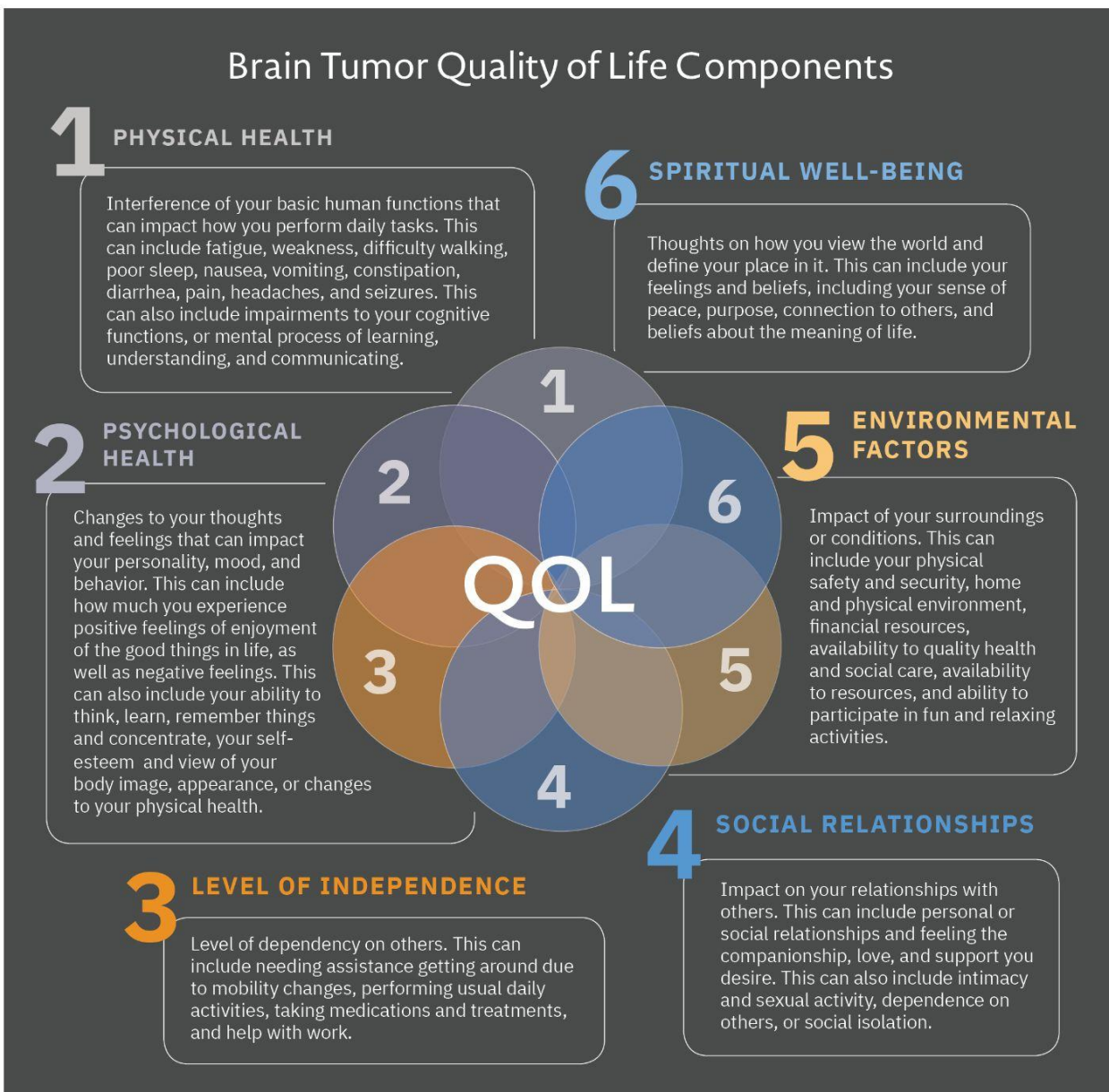
QoL is a primary concern among people living with malignant or non-malignant brain tumors as well as family and other care partners who experience their own QoL needs as they care for their loved ones.

During the stakeholder advisory process, the group built upon existing QoL definitions² to create a definition tailored to the unique needs of the brain tumor community:

Quality of life may be defined as an individual's sense of well-being and ability to enjoy and participate in life. QoL includes physical and psychological health, level of independence, social relationships, environmental factors, and spiritual well-being. QoL is defined by the individual and can evolve over time.

The advisory group recommended that the definition above be supplemented with additional details, as shown in Figure 2.

FIGURE 2



² *The World Health Organization Quality of Life (WHOQOL):*
<https://www.who.int/publications/i/item/WHO-HIS-HSI-Rev.2012.03>

Quality of Life - NCI Thesaurus:
https://ncit.nci.nih.gov/ncitbrowser/ConceptReport.jsp?dictionary=NCI_Thesaurus&ns=ncit&code=C17047

Quality of Life - NCI Dictionary of Cancer Terms:
<https://www.cancer.gov/publications/dictionaries/cancer-terms/def/quality-of-life>

After building a solid foundation by defining QoL and identifying the components of QoL, NBTS and the stakeholder advisory group were able to identify key areas of focus for future research, including recommendations for how QoL programming might be carried out and identifying patient-centered research methods.

Recommendations for NBTS QoL Programming Over Time

Through an engagement process, the stakeholder advisory group established the following concepts and aims to guide NBTS's QoL efforts into the future:

- Build a strong evidence base for the specific QoL needs of the brain tumor community.
- Increase access to brain tumor-specific QoL services.
- Address disparities and inequities specific to people facing brain tumors.
- Develop and fund patient-centered brain tumor QoL research.
- Promote dialogue and engagement between all stakeholders in brain tumor research.
- Advocate for research funding and policies that prioritize key gaps in current knowledge and services.
- Strengthen collaborative science and the inclusion of brain tumor QoL research in clinical trials.
- Foster alignment with federal and existing research initiatives (i.e., NCCN and COG Guidelines, Cancer Moonshot, National Cancer Plan, PCORI methodologies, professional association initiatives).

Identification of QoL Research Focus Areas

NBTS asked stakeholder advisors for recommendations on which areas of QoL research should be prioritized and funded, focusing on topic areas where there is the most need, where there are gaps, or where there is high potential for research to lead to dramatic or transformative improvements. Through that process, NBTS and community stakeholders outlined five brain tumor QoL research focus areas (Figure 3). Some examples are included under each focus area below.

FIGURE 3



Brain Tumor QoL Focus Areas

QoL Outcomes

Developing and testing domains and measures of QoL outcomes over time among patients with brain tumors and their caregivers/partners for use in clinical trials

- Autonomy and activities of daily living, including meaningful participation in life, how a diagnosis impacts one's family, the need for patient independence with less burden on care partners, and utilization of support groups and survivorship clinics
- Mental health, including emotional and existential distress and the need for anticipatory guidance
- Side effects of drugs and the need to lessen the impact on patients' lives, including reviewing potential risks and benefits, impact on decision-making, and long-term side effects

Interventions to Improve QoL Outcomes

Testing the effectiveness, implementation, and dissemination of health care services and models designed to enhance QoL outcomes

- Palliative care, including providing educational resources on when and what to ask one's doctors, definitions of the term "palliative care," and standardization of a palliative care baseline for persons living with brain tumors (particularly neuro-palliative care)
- Interventions for care partners, family, and other social support people, including determining how symptoms interfere with life roles, change relationships, and affect their sense of identity or life worth

Survivorship Research

Research on understanding, monitoring, and addressing the concerns of survivors throughout their lives

- Tracking long-term outcomes to better monitor continuously rather than only monitoring endpoints, to gather data on patients at a variety of ages, and to determine the side effects of drugs and other issues related to long-term survivorship
- Life transition periods, including those moving from pediatric to adult care settings, and addressing the experience of the patient when not in treatment or at the end of life

Disparities and Health Equity

Research focused on assessing and addressing disparities and promoting equity in QoL

- Disparities, including those related to access to care, impact of care, and outcome by race, gender, gender identity, or genetics

- Access to care issues in urban and rural settings, including evaluating community physician follow-up and execution
- Financial toxicity, including evaluating the availability of financial resources and developing strategies to alleviate the high cost to patients and their families

Special Populations and Specific Needs

Research that addresses the unique needs and diverse contexts of the brain tumor community

- Different tumor types
- Age groups
- Caregivers, siblings, other family

Key Topics

As a result of the stakeholder process, NBTS and community stakeholders identified key topics where there are unanswered research questions that address QoL in pediatric, adolescent, young adult and adult brain tumors across the five areas identified above focusing on the patient and caregiver/partner:

- **Adolescent and Young Adult-Specific Priorities**, such as education, close relationships, intimacy, and fertility as well as career pathways and life skills
- **Acute and Ongoing Management** of symptoms, side effects, and cognitive and physical deficits, especially related to fatigue
- **Behavioral and Health Care**, focused on mental health, depression, anxiety, coping and caregiver burden and distress, nutrition, exercise, sleep, and complementary therapies
- **Clinical Trials to Test QoL Interventions** and more consistent integration of QoL measures including, but not exclusive to patient reported outcomes, into clinical trials for patients and caregivers/partners
- **Cognitive Rehabilitation**, neuro-cognitive intervention and evaluations
- **Others Components of QoL**, including environmental factors and spiritual well-being (see Figure 2)
- **Patient and Caregiver/Partner Education and Informational Resources**, including “toolkits” and “roadmaps” with anticipatory guidance and early intervention
- **Patient and Family Navigation**, especially during transition periods (newly diagnosed, recurrence, life transitions, continuum of care, and end of life)
- **Patient Care and Access to Resources**

- **Pediatric and Family-Specific Concerns**, including long-term risk/benefit of treatment, long-term impacts, education, and effects on family relationships (including siblings)
- **Primary [Palliative Care](#)**, including early interventions and rebranding and reconceptualization of what constitutes palliative care, when it is recommended, and who comprises the team
- **Provider and Researcher Education**, including education for non-brain tumor providers and schools on the diverse and idiosyncratic experience of brain tumors, clinical trial conduct guidance, and opportunities for fostering collaborative relationships in both clinical care and research
- **[Survivorship](#)**, including identity, autonomy, and function, ability to work, educational needs, adaptive support, and longitudinal assessment of QoL
- **Transportability and Usability of Data and Health Information**, including access to data and medical records, all in one place and optimization for research

[See Appendix A: Considerations for designing a QoL research project.]

Forward Directions

With gratitude to the QoL stakeholder advisory group, the broader group of participants in the stakeholder meetings, as well as PCORI, NBTS is prepared to consider the findings from this stakeholder engagement process as we develop future funding opportunities for QoL research. In addition, NBTS aims to make this agenda public so that other organizations may use it as the basis of their own work related to QoL research so that the broader brain tumor community can work effectively and collaboratively to improve research, and recognize that QoL is vital to patients and care partners.

The Brain Tumor QoL Research Agenda was been designed to encourage, a) engagement of patients, caregivers, care partners, researchers, health care providers, non-profit organizations, funders, and policymakers, and b) investment in resources to build on the evidence for brain tumor QoL through rigorous, patient-centered outcomes research. Stakeholders will continue to be engaged to advance the field, such that:

- Patients and caregivers/care partners can learn about QoL research efforts and identify ways to participate in this initiative. In addition, volunteer research advocates are paramount to the identification and co-design of QoL research.

- Researchers can inform study design, identify and include QoL research in existing research efforts, and increase engagement with research advocates (i.e., patients) in the development of study design and implementation.
- Health care providers can identify strengths and gaps in existing care, support the development and collection of brain tumor QoL data, and foster patient-centered care, and patient, family, and clinician education on brain tumor QoL needs.
- Health care, non-profit, and community-based organizations can prioritize opportunities for brain tumor QoL research, identify collaborative relationships and partnership opportunities to establish and increase brain tumor QoL research, collaborate on policy and advocacy opportunities to increase QoL research funding, and establish norms for patient-centered QoL research and engagement.
- Insurers (public and private), policymakers, and funders can inform policy, coverage, and funding to increase access to QoL services.

Thanks to funding from PCORI, NBTS had the valuable opportunity to engage in a comprehensive and conclusive process, from stakeholder identification through the development of its research agenda. For NBTS, patients and care partners are our priority, and we will continue to fund research that is critically needed to improve survival and QoL for patients and care partners.

NBTS would like to thank stakeholders for their feedback and participation. Learn more about the [Quality of Life Research Project](#) and the stakeholder advisory group members.

This program was funded through a Patient-Centered Outcomes Research Institute® (PCORI®) Eugene Washington PCORI Engagement Award (EACB-23261).

Appendix A: Considerations for Conducting QoL Research

Core Values

- **Patients and Families First:** Fiercely committed to providing a voice and community for all individuals impacted by brain tumors.
- **Best-in-class Science and Service to our Community:** Adhering to rigorous methodology standards across scientific and community initiatives - seeking innovation while disseminating and implementing existing evidence and resources.
- **Collaborative and Inclusive:** Including patients and other stakeholders at all stages of the research process - families, care partners, researchers, clinicians, industry, and policymakers - from conceptualization to conduct, to the dissemination of innovations to those who stand to benefit. Working with others is critically important to achieving the mission and ensuring the equitable impact of research.
- **Promoting Health Equity and Eliminating Disparities in Access to High-quality, Patient-centered Care:** Ensuring that diverse voices are respected and valued is critical to effectively uniting the brain tumor community, advocating for change, removing barriers to health care access, and supporting innovation in research.
- **Early Intervention and Preservation of Autonomy and Function:** Focusing on preventing side effects and changes in autonomy, body functions and activities of daily living, or tackling them when they occur or get worse.

Types of Research

- **Patient Centered Outcomes Research (PCOR)** PCOR focuses on outcomes most important to patients. PCOR findings can help patients and other health care stakeholders, such as caregivers, clinicians, insurers, and policy makers, make better-informed decisions about their health and health care options.³
- **Comparative Effectiveness Research-(CER)** CER compares the effectiveness of two or more interventions or approaches to health care, examining their risks and benefits. CER findings assist clinicians, patients, and other stakeholders in making informed decisions that improve health care. Comparing two or more interventions distinguishes CER from other types of clinical research. It can both validate a particular intervention and identify which treatments best meet a certain population's needs.⁴
- **Dissemination and Implementation Science (D&I):** D&I science refers to “the systematic study of processes and factors that lead to widespread use of an evidence-based intervention by the target population” (dissemination research) and “the processes and factors that are associated with successful integration

of evidence-based interventions within a particular setting” (implementation research).⁵

Types of Study Methodologies

Investigators are encouraged to use any and all appropriate research methodologies to answer priority research questions, including observational research, randomized trials, and use of qualitative, quantitative, and mixed methods:

- **QoL Measurement** - development, adaptation, and use of measures for assessing QoL, including appropriate measure development and validation methods and/or patient-centered technologies for collecting and using high quality, valid, and reliable data on brain tumor QoL in research.
- **Evidence-Based Intervention Research** - development, pilot and feasibility trials, and full-scale randomized trials of interventions, programs, service models, or other techniques designed specifically to improve QoL in people impacted by brain tumors, either in general or focused on particular components as prioritized by the brain tumor community of interest).
- **Dissemination and Implementation Research** - assessment of barriers and facilitators to adoption, implementation, and sustainability of effective programs, services, and techniques for enhancing QoL for people impacted by brain tumors; developing and testing dissemination and implementation strategies including policy development and advocacy research; enhancing equitable impact of interventions on brain tumor community outcomes.

³ *Patient-Centered Outcomes Research Institute (PCORI)*

⁴ *Patient-Centered Outcomes Research Institute (PCORI)*

⁵ *Rabin, Viglione, & Brownson, Terminology for Dissemination & Implementation Research, 2023*
