National Brain Tumor Society

June 28, 2017

Streamlining Clinical Trials to Accelerate Brain Tumor Drug Development

Roundtable participants include clinicians, researchers, representatives of biopharmaceutical companies, patients, patient advocates, and regulators.

PURPOSE: For the inaugural Research Roundtable, participants discussed how novel endpoints and novel, innovative designs can optimize primary brain tumor clinical trials for investigational therapeutics.

Key topics included:

- Potential utilization of radiographic endpoints, including tumor volume growth rate, with clinical outcomes assessment to demonstrate efficacy.
- Feasibility of using novel biomarkers such as the assessment of steroid use and rate of seizures as surrogate endpoints and early indicators of clinical benefit.
- Novel trial design for brain tumor clinical trials, including master protocols and adaptive randomization.
- How to potentially incorporate real-world evidence to inform the development of brain tumor treatments.

Major conclusions included:

- To advance the development of novel clinical trial endpoints, the neuro-oncology field will need to generate data that supports that the proposed new endpoints are meaningful to patients.
- More robust early-stage drug development is needed, as is a focus on the potential for repurposing drugs for use in treating brain tumors.
- Early surrogate markers of treatment effects could provide confidence in moving forward into phase II trials, but they need to be validated and linked to measures that adequately assess how a patient feels or functions if the endpoint is intended to be used to support drug approval.
- In assessing outcomes from trials in diffuse low-grade gliomas, there is a significant gap in knowledge about tumor growth rates. More data on natural history, in molecularly

defined groups, is key to advancing new endpoints and defining priorities for the use of clinical outcome assessments in these trials.

- There is an opportunity to conduct more efficient trials, leveraging partnerships between the patient advocacy community, researchers, industry, and regulators.
- Real-world evidence provides an opportunity to incorporate patient experiences in drug development and improve decision making. This trend presents a “call to action” for the brain tumor community to establish systems and a framework that will enable the community to build “game-changing” pragmatic clinical trial networks.