



**December 12, 2017**

## **Improving Early Phase Clinical Trials to Accelerate Brain Tumor Drug Development**

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

**PURPOSE:** To identify action steps that will increase and enhance efforts to more rigorously qualify the most promising clinical candidates in early-phase testing for prioritization and advancement to later phase brain tumor trials. This topic was one of the key areas identified by the June 2017 Research Roundtable as a priority for the field.

### **Key topics included:**

- Prioritizing what the brain tumor community needs from early phase trials (including alignment on approaches for definitions, measurement, and trial design) and how to best measure key properties of an agent during this stage of research.
- Defining critical components that can inform sponsor evaluation for go/no go decisions in moving a compound into later stage development.
- Defining actions needed to develop a fully-integrated early phase research network.

### **Major conclusions included:**

- There are multiple challenges in brain tumor drug development, including:
  - Lack of preclinical data
  - Lack of understanding of blood-brain barrier penetration
  - Frequent lack of recommended phase II dose/need for additional phase I/II studies
  - Small size of the GBM market, and poor history of success in drug development for GBM
- These challenges can be addressed through rapid, efficient preclinical evaluation, allowing the most promising novel agents to be selected for clinical testing and evaluated according to a standard set of criteria and thresholds.

- There is consensus on the goal of key stakeholders from across the neuro-oncology ecosystem working together to bridge these gaps, mitigate barriers, and reduce risks so that industry (large and small companies) can bring drugs to brain tumor patients.
- There is broad interest in defining a proof-of-concept/pilot study that could demonstrate the benefits of an early phase clinical trial network/infrastructure.