

## **BMSC Strategic Priorities**

### **September 2018**

The BMSC general Strategic Priorities are as follows:

1. **Biologically or genomically based trial designs**, which take into consideration **molecular subsets of tumors** (e.g., MGMT methylated or unmethylated GBMs, 1p/19q codeleted gliomas, GBMs with molecular alterations that predict potential sensitivity to a novel agent (INSIGHT trial), SHH or beta catenin subsets of medulloblastomas in children and adults, BRAF-targeted therapies for pediatric LGG and HGG), in which case the **biomarkers are integral** to the study design.
2. Studies that **pair administration of a novel agent (e.g., small molecule) or modality (e.g., immunotherapy), with pharmacodynamic or immunological measures or tissue analyses of the drug target and studies of drug penetration into the tumor**, such that **mechanistic hypotheses** directly relevant to the agent/modality in question can be tested that may form a foundation for subsequent trials.
3. Studies that pair novel agents or modalities with **imaging biomarkers or molecular biomarkers** as hypothesis-testing or hypothesis-generating tools.
4. Studies that focus on “**process improvement**” approaches that may enhance QOL and/or influence outcome (e.g., whether temozolomide adds benefit post-RT vs. only during RT; use of proton vs. photon irradiation). Since these studies focus on process improvement, non-conventional endpoints could well drive the design of these trials (for example, decreasing the subsequent burden of therapeutic intervention, after a given test or study intervention, as compared to standard-of-care therapy).
5. **Innovative/new approaches including:**
  - **Combinatorial approaches**, including various combinations of standard therapies (e.g. radiotherapy, chemotherapy, etc.) with novel and emerging therapies, ranging from phase 0 to phase 3 trials.
  - **Translation of promising early phase results** from any potential source, within or outside the NTCN network, using the robust framework of large, definitive randomized trials.
  - Studies that address critical unmet needs in adult and pediatric neuro-oncology, especially for tumor types with extremely poor survival or in disease subsets where the ‘standard’ treatment results in unacceptable quality-of-life/functional outcomes.