

NCI BMSC Strategic Priorities:

Preamble:

Present treatments for many forms of adult and childhood brain tumors result in poor overall survival, an excessive degree of neurotoxicity, and other unacceptable outcomes, including poor quality-of-life. The BMSC will prioritize studies utilizing agents with novel mechanisms of action, novel approaches with existing therapies, innovative means to better deliver agents to the brain, and studies integrating informative biomarkers and neuro-imaging investigations to accelerate the development of more effective, less toxic therapies. Accordingly, the following priorities are considered equally important.

Strategic Priorities:

Priority #1: Trial designs based on disease biology and genomics taking into consideration biologically enriched patient populations harboring actionable genomic alterations in both adults and children.

Priority #2: Hypothesis testing and hypothesis generating studies that pair administration of novel agents or modalities with imaging or molecular biomarkers.

Priority #3: Studies that pair novel agents or modalities with pharmacokinetic, pharmacodynamic, immunological, imaging, and biospecimen analysis of the drug target. Simultaneous or early development studies of drug penetration and drug effect in the tumor, are encouraged.

Priority #4: Studies that focus on modifications of the current standard therapies to enhance efficacy, QOL, reduce toxicity, and enhance other clinical outcomes are encouraged. Novel endpoints can be studied, used and validated in such clinical trials.

Priority #5: Innovative combinations of standard therapies with novel therapies, ranging from phase 0 to phase 3 trials. This includes 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.