

2015 Strategic Priorities

Pediatric and Adolescent Solid Tumor Steering Committee (PASTSC)

The overall goal of the NCTN is to perform definitive phase 3 trials. Phase 3 trials are difficult to do therefore proposals for studying agents in the phase 3 setting should be based on agents that are active in the phase 2 setting or in model systems or there is compelling basic and translational science to support a strong rationale in the targeted disease.

Bone Tumors (Osteosarcoma and Ewing Sarcoma)

- 1) Evaluate new agents through a series of phase 2 trials, including when feasible randomized phase 2 trials, in the relapsed or high-risk (e.g. metastatic) settings to identify agents of interest to test in the phase 3 setting. Agents and combinations will be selected based on mechanism of action or compelling basic and translational science studies suggesting a strong rationale, evidence from model systems, and available clinical data. Agents with novel mechanisms (as compared to standard therapy) are a priority in investigations.
- 2) Evaluate the integration of promising new agents with activity against osteosarcoma and Ewing sarcoma into a multi-modal backbone to improve outcome in each of the diseases.
- 3) Evaluate surgical approaches and outcomes for local control of metastatic disease.
- 4) Evaluate biomarkers and interventions for patients predicted to have a poor outcome at diagnosis or high risk of recurrence. Continued tissue collection is essential to advance biomarker determinations for osteosarcoma and Ewing sarcoma and focused new tissue collection may be warranted to access unique tumor (such as metastatic and/or relapsed lesions)

Neuroblastoma

- 1) Evaluate the integration and optimization of clinically rational targeted therapies (e.g. MIBG, immunotherapy, ALK inhibitors) into a multi-modal backbone to improve outcome for high-risk neuroblastoma.
- 2) Identify biomarkers and evaluate novel interventions for patients predicted to have a poor response to treatment or disease progression.
- 3) Evaluate the addition of molecularly and cellularly targeted therapy for relapsed neuroblastoma that ultimately can be applied to newly diagnosed patients.
- 4) Evaluate treatment approaches associated with a reduction or elimination of therapy that will improve safety yet maintain efficacy for patients with biologically favorable neuroblastoma.
- 5) Optimize biologic specimen collection as a key aspect in determining biomarkers and targets in neuroblastoma.

Soft Tissue Sarcomas

- 1) Evaluate new agents through a series of phase 2 trials, including when feasible randomized phase 2 trials, in the relapsed or high-risk (e.g. metastatic) settings to identify agents of interest to test in the phase 3 setting. Agents will be selected based on mechanism of action, evidence from model systems, and available clinical data.
- 2) Validate functional and molecular markers (e.g., circulating tumor DNA) that may be associated with outcome or risk classification. Tissue collection is essential to achieve these goals in soft tissue sarcomas.
- 3) Collaborate with other NCTN groups to identify soft tissue sarcoma subgroups of interest to evaluate new treatment approaches.