

C3 / Padres Pedal the Cause 2020

“A pilot trial to test the benefit of real time drug screening to determine individualized treatment plans for children and young adults with relapsed or refractory medulloblastoma”

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SCIENTIFIC ABSTRACT

Medulloblastoma (MB) is the most common malignant brain tumor in children. Although significant advances have been made in treating MB, ~30% of patients still die from the disease, and survivors suffer severe long-term side effects from therapy. Patients who relapse have extremely poor outcomes, and new approaches to treating them are particularly important. In this proposal, we seek funding for a unique pilot clinical study in which we combine genomic analysis and high-throughput drug screening to identify more effective therapies for relapsed MB patients. Our previous studies have demonstrated the feasibility of performing DNA sequencing and drug screens on patient samples, and have shown that these analyses can provide insight into possible therapies. Our pilot study will test whether this type of analyses can be done in a clinical setting (in CLIA-certified labs), whether results can be used by a molecular tumor board to make recommendations for therapy, and whether these recommendations are beneficial for patients. If our approach is successful it will improve outcomes for relapsed MB patients and provide a new paradigm for personalized therapy of other pediatric cancers.

LAY ABSTRACT

Medulloblastoma is the most common malignant brain tumor in children. Although significant advances have been made in treating medulloblastoma, many patients still die from the disease, and those who survive suffer severe long-term side effects from therapy. Patients who relapse have extremely poor outcomes, and new approaches to treating them are particularly important. We propose to carry out a unique clinical trial in which we take tissue from a patient's tumors at the time of surgery, sequence it to identify mutations that may be causing the tumor, and also test the sensitivity of tumor cells to drugs that have been used against other cancers. Using this approach, we hope to identify more effective therapies for each patient. Our previous studies have demonstrated the feasibility of performing sequencing and drug testing on patient samples, and have shown that these analyses can provide insight into possible therapies. Our proposed trial will test whether this type of analyses can be done in a clinical setting, whether results can be used by a panel of experts to make recommendations for therapy, and whether these recommendations are beneficial for patients. If our approach is successful it will improve outcomes for relapsed MB patients and provide a new paradigm for personalized therapy of other pediatric cancers.