

RAD001 Clinical Trial Summary

Low-grade gliomas are the most common brain tumor in children. Although some patients can be effectively cured with surgery alone, recurrences are common in a significant majority of patients—especially those without neurofibromatosis type I (NF1). These patients often require repeated resections, chemotherapy and often radiation therapy. The damage caused by these therapies has a significant impact on the lives of these children. The need for more targeted and less toxic therapies in this population are therefore required. An important pathway that has been implicated in pediatric low-grade gliomas is mTOR, a central relay site within the cell that when activated, results in increased proliferation, cell migration and angiogenesis. Our preliminary work has demonstrated the presence and activation of this pathway in LGG samples from children without NF1. RAD001 is a new oral mTOR inhibitor that has demonstrated excellent inhibition of this pathway at clinically achievable doses. The drug is exceedingly well tolerated and is currently used to reduce the risk of solid organ transplant rejection. We are now proposing a formal multi-institutional clinical trial of RAD001 in non-NF1 children with recurrent or progressive LGGs after standard treatment.

Written by Dr. Mark Kieren, pediatric oncologist, Dana Farber Cancer Institute for the Childhood Brain Tumor Foundation, Germantown, MD.