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MicroRNAs (miRNAs) are a recently discovered class of tiny non-coding regulatory RNA molecules. By regulating expression of protein-coding genes, microRNAs play critical roles in development, growth, cell proliferation, and lineage determination. At least several hundred unique microRNAs are expressed in humans and these microRNAs are estimated to affect the expression of more than 30% of human proteins. By screening about 200 microRNAs expressed in human brain, we have recently identified several of those whose expression is elevated in high-grade brain tumors. During the last year, we have developed technologies to suppress these microRNAs in cultured glioblastoma and medulloblastoma cells. Knockdown of one of these microRNAs, miR-21, in glioblastoma cells triggered activation of caspases (enzymes mediating cell death) and therefore led to increased cell death. Our data suggest that miRNAs may represent a novel class of therapeutic targets for the treatment of malignant brain tumors.

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"MicroRNAs targeting in medulloblastoma"

Dr. Krichevsky aims to treat medulloblastomas by targeting microRNAs. MicroRNAs (miRNAs) are a novel class of small non-coding RNA molecules that have been shown to regulate gene expression. These miRNAs are thought to play critical roles in the control of growth, proliferation, development and determination of cell lineage. A cluster of microRNAs have shown to be elevated in both medulloblastomas and glioblastomas. This research may lead to the development of novel treatments by targeting miRNAs to help improve the biology of pediatric brain tumors. This study was funded by the support of all of our donors and we offer special thanks to the Schiller family for the added contribution from Amy's Run that contributed strongly to one year of funding for this two-year project.

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