Pediatric Phase II Trials of Poly-ICLC in the Management of Newly Diagnosed and Recurrent Brain Tumors.


Abstract
Brain tumors are the most common solid tumor diagnosed in childhood that account for significant morbidity and mortality. New therapies are urgently needed; hence, we conducted the first ever prospective open-label phase II trials of the biological response modifier, poly-ICLC, in children with brain tumors. Poly-ICLC is a synthetic double-stranded RNA that has direct antiviral, antineoplastic, and immune adjuvant effects. A total of 47 children representing a variety of brain tumor histopathologic subtypes were treated with poly-ICLC. On the basis of the results of the initial phase II trial, an expanded prospective phase II trial in low-grade glioma (LGG) has been initiated. MRI was used to acquire volume-based measures of tumor response. No dose-limiting toxicities have been observed. In the initial study 3 of 12 subjects with progressive high-grade gliomas (HGGs) responded, and 2 of 4 children with progressive LGG experienced stable disease for 18 to 24 months. In the follow-up LGG phase II study, 2 of 5 LGG patients were stable over 18 months, with 1 stable for 6 months. Overall 5 of 10 LGG patients have responded. On the basis of low toxicity and the promising LGG response, poly-ICLC may be effective for childhood LGG, and the results justify biomarker studies for personalization of poly-ICLC as a single agent or adjuvant.

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