Brainstem Tumors in Neurofibromatosis Type 1

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It's well-known that 15-20% of young children with NF-1 will develop an optic pathway tumor at a young age. These tumors typically come to medical attention when they present either with reduced visual acuity discovered on the recommended yearly eye exams for all young children with NF-1 or due to symptoms of the early onset of puberty such as rapid growth at a young age. Optic pathway tumors may affect the optic nerves, the optic chiasm which is the part of the brain where the two nerves intermingle, or the optic tracts and optic radiation which are behind the optic chiasm.

However, tumors may develop in other parts of the brain, particularly the brainstem. Brainstem tumors represent the second most common brain tumor occurring in children with NF1. The brainstem is the back part of the brain which connects the brain to the spinal cord. Among other functions, it controls our breathing, swallowing, heart rate, blood pressure and level of consciousness. Even very small tumors in the brainstem can have significant effects on any of these functions.

Although brainstem tumors may develop at any age, they tend to be seen in younger children at an average age of 7 years. Symptomatic tumors may come to medical attention in several different ways. Most commonly, the tumor creates a blockage of the fluid that surrounds the brain, cerebrospinal fluid. This creates a buildup of pressure leading to a condition called hydrocephalus. Children who have hydrocephalus will experience nausea, vomiting and severe headaches particularly at night or early in the morning. Other children may present with such diverse problems such as swallowing difficulties, a new onset of nasal speech or disturbances in the ability to walk or maintain balance.

Despite the above, more than half the children with NF-1 brainstem tumors are totally asymptomatic at the time of diagnosis. These tumors are often discovered in children undergoing MRI scans for different reasons such as nonspecific, unrelated headaches or for follow-up of an optic pathway tumor. Most often brainstem tumors in NF-1 are benign low grade gliomas, similar to the optic pathway tumors these children may develop. They are rarely cancer and do not spread to other parts of the body. While these tumors will almost always continue to grow in children who do not have NF-1, NF-1 tumors

may never grow or require any treatment. In a recent large multicenter study, only 41% of NF-1 brainstem tumors required some form of therapy.

An extremely important point to remember is that MRI scans in children with NF1 frequently reveal non-enhancing T2 hyperintense lesions, sometimes called "unidentified bright objects". UBOs are radiographic anomalies found in over half of children with NF-1; they never cause a problem and disappear over time. Unfortunately, they are often interpreted as being tumors by inexperienced clinicians and neuroradiologists. This is one important reason why all children with NF-1 associated brain tumors of any type need to be cared for by experts who have significant NF-1 experience.

NF-1 brainstem tumors are infrequently biopsied or removed because of their precarious location. On occasion, tumors confined to the cerebellum (the part of the brain that controls balance) may be removed in their entirety without the aid of any further therapy. Tumors that produce hydrocephalus may require the placement of a ventriculoperitoneal shunt which allows the fluid to bypass the blockage created by the tumor. This may be the only treatment ever required. Remember many of these tumors will never grow or require any form of treatment. However effective therapy is available with chemotherapeutic agents such as carboplatin and vincristine or possibly the newer class of drugs, MEK inhibitors.

In summary, if your child receives a diagnosis of a brainstem tumor, don't panic. It may never grow or cause any problems. Even if treatment is required, they are infrequently malignant and definitely treatable. Most importantly, if you're not yet under the care of a NF-1 expert physician or clinic, this would be the time to seek one out.