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Proton therapy shows efficacy, low toxicity in large cohort of children with high-risk neuroblastoma

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Researchers from Children's Hospital of Philadelphia (CHOP) and the Perelman School of Medicine at the University of Pennsylvania analyzed the largest cohort to date of pediatric patients with high-risk neuroblastoma treated with proton radiation therapy (PRT), finding both that proton therapy was effective at reducing tumors and demonstrated minimal toxicity to surrounding organs.

The study is published online in the *International Journal of Radiation Oncology*.

"These data are extremely encouraging and could be a game-changer for a number of reasons," said lead author Christine Hill-Kayser, MD, Chief of the Pediatric Radiation Oncology Service at Penn Medicine and an attending physician at CHOP. "Not only did we observe excellent outcomes and minimal side effects that validate the use of PRT in high-risk neuroblastoma patients, we answered a lingering question about proton therapy—the concern that because it is so targeted, tumors may come back. Tumors mostly did not come back—suggesting PRT is effective, less toxic and a superior choice for our young patients who must endure intense treatment modalities in an effort to cure this high-risk cancer."

Neuroblastoma is the most common cancer in infants, accounting for more than 10 percent of all childhood cancer deaths. Primary

neuroblastoma tumors are commonly adrenal tumors, which are very close to the kidney, liver, pancreas and bowel in children, making them hard to treat without harming vital organs in tiny bodies. Treatment usually involves a combination of therapies including chemotherapy, radiation and surgery.

Researchers studied 45 patients with high-risk neuroblastoma who received PRT at both institutions between 2010 and 2015. CHOP cancer patients who need radiation therapy are treated at Penn Medicine, including PRT through the Roberts Proton Therapy Center.

Unlike traditional photon radiation using x-rays, PRT is a non-invasive, precise cancer treatment that uses a beam of protons moving at very high speeds to destroy the DNA of cancer cells, killing them and preventing them from multiplying. Highly targeted, PRT has significant promise for treating tumors in very young children and may reduce radiation exposure to healthy, developing tissue that may result in lifelong impacts.

Five years after treatment, the longest recorded period of study in the largest cohort of patients to date, researchers observed excellent outcomes, with 82 percent of patients still alive, and 97 percent free of a primary site tumor reoccurrence.

Toxicities, or side effects, are measured on a scale from 1 to 5, with 5 being the most severe. No patient observed in the study experienced grade 3 or 4 long-term acute liver or kidney toxicity, with the majority of patients experiencing grade 1 side effects from PRT. "We've showed PRT is not only effective in the treatment of high-risk neuroblastoma, but it also spared damage to the developing liver, kidneys and bowel that may occur in pediatric patients exposed to traditional radiation," said Hill-Kayser. "While we look forward to longer-range data on these patients 10 years down the road, the excellent outcomes we see here,

coupled with the fact the precision proton approach did not increase recurrence rates, support the expanded use of proton therapy in neuroblastoma and other high-risk childhood cancers." Additional studies with extended follow-up and larger patient numbers are planned. The Cancer Center at Children's Hospital of Philadelphia offers one of the most established and experienced pediatric proton radiation therapy programs, in collaboration with Penn Medicine at the Roberts Proton Therapy Center. For more information, please click here: Pediatric Proton Therapy Center.

Provided by Children's Hospital of Philadelphia