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Prospective study finds many children with retinoblastoma can safely forego adjuvant chemotherapy

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New results from a prospective clinical trial conducted in France show that children with low-risk retinoblastoma do not need postoperative (adjuvant) chemotherapy to prevent disease recurrence or metastasis; the results also suggest that certain patients with intermediate-risk disease can receive less aggressive adjuvant treatment, or perhaps forego it altogether. Avoiding chemotherapy spares patients from treatment side effects and long-term health risks, such as cardiovascular disease and development of a second cancer.

Retinoblastoma is a rare form of cancer, accounting for three percent of all cancers diagnosed in children under the age of 15. An estimated 300 cases of retinoblastoma will be diagnosed in the United States this year, most before the age of five years. The tumor begins in the retina, a thin layer of nerve tissue that coats the back of the eye.

About two thirds of children with retinoblastoma have unilateral disease, meaning that only one eye is affected by the tumor. In developed countries, this type of disease is typically detected around two years of age. More than 95 percent of such patients are cured by surgical removal or the affected eyeball, with eyesight preserved in the other eye. However, some patients remain at risk of disease spreading and recurrence.

To estimate the risk of disease recurrence and metastasis, doctors

examine the surgically removed eye under a microscope and classify the tumor as either low-, intermediate-, or high-risk. All patients whose tumors are considered high-risk receive intensive postoperative chemotherapy and radiation therapy. There is no general consensus, however, on the need for adjuvant therapy and the optimal treatment approach for patients in the other two risk categories. Therefore, protocols for adjuvant chemotherapy use in these patient groups have varied widely.

"Our study confirms that it is not necessary to give adjuvant treatment to many children with unilateral retinoblastoma," said lead study author Isabelle Aerts, MD, a pediatric oncologist at Institut Curie in Paris, France. "I think that our results will help establish the standard of care for such patients, reducing variations in postoperative chemotherapy protocols."

In the present study, 123 patients with unilateral, non-hereditary retinoblastoma were assigned to one of three risk groups according to previously established criteria. The median age at the time of eye removal surgery was 23 months. The 70 patients with low-risk disease received no postoperative chemotherapy; the 52 patients with intermediate-risk disease received four courses of adjuvant chemotherapy (etoposide, carboplatin, vincristine, cyclophosphamide), and the only patient with high-risk disease received six courses of high-dose adjuvant therapy (etoposide, carboplatin, thiotepa, vincristine, and cyclophosphamide).

At a median follow-up period of 71 months, all patients were alive. None of the patients experienced disease worsening, relapse, distant metastasis or secondary cancers. The adjuvant chemotherapy was safe overall. These data confirm that the specific regimens administered in this study, which are already being used around the world, are very effective and safe.

The excellent outcomes observed in this study confirm it is safe for patients with low-risk disease to go without adjuvant therapy and suggest that it may be safe to reduce the intensity of or even eliminate adjuvant chemotherapy in some patients with intermediate-risk disease, specifically those with a risk feature known as isolated choroidal invasion—spreading of tumor cells to the layer of blood vessels and connective tissue at the back of the eye. However, more research is needed to establish clinical criteria for identifying such patients. As all patients were alive at the end of the follow up period, it is not clear whether adjuvant treatment played a vital role, or if surgery would have been sufficient.

Aerts and her research team have recently launched a new study to confirm these results and also determine if treatment could be reduced in some patients in the intermediate-risk category. In this ongoing study, patients with intermediate-risk disease and isolated choroidal invasion are being treated with only two cycles of adjuvant chemotherapy.

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