ここ30年における横紋筋肉腫治療の一番の進歩 (Abstract LBA2)

維持化学療法は小児横紋筋肉腫の生存期間を延長する

Maintenance chemotherapy extends life for children with rhabdomyosarcoma

新たな化学療法戦略は、再発リスクの高い小児横紋筋肉腫の治癒率を改善する、と2018 ASCO Annual Meeting Plenary Sessionで取り上げられた。標準的な初期治療完遂後、371人が治療中止（以前の標準治療）群または低用量ビノレルビン静注およびシクロホスファミド経口投与6か月施行群に、ランダムに割り付けられた。診断後5年時点における無病生存率は、標準治療群の68.8%に対し維持療法群では77.6%であり、全生存率はそれぞれ73.7% 対 86.5% であった。

Full Text

A new chemotherapy strategy improves cure rates for children with rhabdomyosarcoma who are at high risk for cancer recurrence. In a randomized phase III clinical trial, adding six months of low-dose maintenance chemotherapy after initial treatment increased the 5-year overall survival rate from 73.7% to 86.5%. Children with rhabdomyosarcoma who are alive at five years are considered cured, as tumor recurrence is very rare.

These findings were presented in ASCO's Plenary Session, which features four studies deemed to have the greatest potential to impact patient care, out of the more than 5,800 abstracts featured as part of the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting.

"We have been treating rhabdomyosarcoma the same way for more than 30 years, and although different approaches have been tried, this is the first randomized trial in rhabdomyosarcoma to show improved outcomes. By using existing medicines in new ways, we are establishing a new standard of care and, most importantly, we're helping children and young adults with this rare cancer live longer, with less risk of their cancer returning," said lead study author Gianni Bisogno, MD, PhD, a professor at the University Hospital of Padova in Italy and Chair of the European Paediatric Soft Tissue Sarcoma Study Group.

Rhabdomyosarcoma originates in the muscle tissue and can occur in any part of the body, but it is most often found in the head, neck, pelvis, and abdomen. Rhabdomyosarcoma is rare, accounting for 4% of all childhood cancers.

The prognosis for rhabdomyosarcoma is generally good – 80% of children can be cured with modern treatment, which includes high-dose chemotherapy, radiation, and surgery. However, among children who have metastasis at diagnosis or a recurrence after initial treatment, only 20-30% can be cured.

This trial enrolled patients 6 months to 21 years of age who were considered at high risk for recurrence due to having large tumors located in a part of the body that is difficult to treat (e.g., the head).

After completing the standard initial treatment, 371 patients (79% of whom were 10 years old or younger) were randomly assigned to either stop treatment (the former standard of care) or receive six months of maintenance therapy with low doses of two chemotherapy medicines (intravenous vinorelbine and oral cyclophosphamide).

At five years from diagnosis, the disease-free survival (defined as five years without tumor recurrence or death from any cause) was 68.8% in the standard treatment group vs. 77.6% in the maintenance group, and overall survival rates were 73.7% vs. 86.5%, respectively.

The most common side effect in the maintenance group was low blood cell count, though it was usually mild. Febrile neutropenia occurred in 25% of patients. Infection rates were much lower with maintenance treatment than after initial standard chemotherapy, and neurologic side effects resolved after treatment ended. However, as with most kinds of chemotherapy, long-term side effects are still possible and patients will continue to be monitored.

"By keeping the pressure on this cancer longer with maintenance therapy, we are giving patients two wins – we are boosting cure rates by preventing relapses and doing so with few serious side effects. After three decades of research, this finding goes to show that we will continue innovating treatment, no matter how long it takes," said ASCO Expert Warren Chow, MD.

The findings of this trial have already changed the standard of care in Europe, where investigators shared the results with soft tissue sarcoma study group institutions in 14 countries.

This study received funding from Fondazione Città della Speranza, Italy.

ASCO2018特集