Increased survival with new treatment for aggressive form of childhood cancer

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An international team of scientists, including researchers from Karolinska Institutet and Karolinska University Hospital, have conducted a clinical study on a new treatment for high-risk neuroblastoma, an exceedingly aggressive form of cancer that only develops in babies and young children. According to the researchers, the results demonstrate that the new treatment both increases survival and reduces undesirable effects.

This is a significant step forwards for these seriously ill children," says consultant Per Kogner, professor of paediatric oncology at the Department of Women's and Children's Health, who led the Swedish arm of the study.

The preparation tested is the new pharmaceutical combination busulfan-melphalan, which is given in high doses followed by stem cell transplantation. In all, 1347 children from 18 countries were included in a so called phase 3 study, of which 598 children were randomised to be treated with busulfan-melphalan or standard therapy. The research team found that the survival rate for those who received this new treatment increased by 38 to 50 per cent, while the risk of life-threatening adverse effects decreased from 10 to 4 per cent.

Before the age of two

Neuroblastoma only develops in babies and small children, usually

before the age of two. The tumours arise in the sympathetic nervous system but also often appear inside the adrenal glands and in the spinal cord. Approximately 20 children a year are diagnosed with the disease in Sweden, which means that international cooperation is necessary for there to be studies large enough for researchers to build on in the development of new therapies.

More information: Ruth Ladenstein et al. Busulfan and melphalan versus carboplatin, etoposide, and melphalan as high-dose chemotherapy for high-risk neuroblastoma (HR-NBL1/SIOPEN): an international, randomised, multi-arm, open-label, phase 3 trial, *The Lancet Oncology* (2017). DOI: 10.1016/S1470-2045(17)30070-0

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