Outcome after high-risk neuroblastoma treatment in Europe

Neuroblastoma affects approximately 100 new children each year in the UK. Of these, approximately 50 children are considered to have 'high-risk' disease because their tumour has amplification of the MYC-N gene, or because they are older than 12 months old and the tumour has spread (metastasised).

Although the outcome for this group of children has improved over the last few decades, long-term survival rates from the time of diagnosis are still only around 50%. These current survival rates are achieved with intensive chemotherapy, surgery, stem cell transplant, radiotherapy and anti-GD2 immunotherapy. However, a number of children are unfortunately not able to receive all these treatments, either because their disease does not respond adequately or because they have significant side effects from the treatment.

For those children who do respond well to their treatment, and who have no evidence of remaining disease at the end of treatment (following immunotherapy), the chances of relapse are less than earlier on in treatment. Recent data from the European Neuroblastoma Research Network (SIOPEN) suggests that:

- 85% of children who have successfully completed immunotherapy, and who have no evidence of disease, remain disease-free for the next two years.
- 79% of these children (approximately 4 out of 5 children) who are in remission at both the beginning and end of immunotherapy remain disease free after five years.

A number of US groups are investigating giving children further treatment (for example, a bivalent vaccine and DFMO) at the end of standard treatment, to further reduce the chances of relapse. The outcomes reported to date after receiving either a bivalent vaccine or DFMO are broadly similar to those reported by SIOPEN. Both of these treatments are being tested in a 'non-randomised' way, in that all children receive either the vaccine or DFMO. This makes it impossible to know what impact the DFMO and/or vaccine treatment is having on the outcome. At present, the vaccine and DFMO studies are not designed to answer the important question of whether or not DFMO or vaccine prevents relapse in some children.

SIOPEN, the UK National Cancer Research Institute Neuroblastoma Group and the CCLG Neuroblastoma Special Interest Group are committed to further improving the outcome for children with neuroblastoma.

The next SIOPEN High Risk Neuroblastoma trial will test whether outcomes can be improved by:

i) further intensification of treatment, in the form of a double rather than single stem cell transplant
ii) an increased dose of radiotherapy in patients with residual tumour after surgery
iii) an alternative induction chemotherapy regimen, currently used by the German Paediatric Oncology group

In addition, we are exploring options to investigate whether further treatment following anti-GD2 immunotherapy can reduce relapse rates.

Many parents find the uncertainties at the end of their child's treatment very difficult. This information sheet has been produced to provide the best available assessment of relapse rates for children who successfully complete all standard UK treatment for high-risk neuroblastoma. If you have any questions about any of this information, then we would encourage you to speak to your child's consultant.

CCLG Neuroblastoma Special Interest Group
October 2019