

Targeted therapy could be first line treatment for childhood cancer

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Alexander Mohammed, age 10. Credit: Newcastle University

An international study, involving Newcastle University, has for the first time found a genetic marker in tumors from patients with high-risk neuroblastoma.

Research, published in the *Journal of Clinical Oncology*, has identified that alterations in the [neuroblastoma](#)'s ALK (anaplastic lymphoma kinase) gene are associated with a significantly poorer prognosis for

children with high-risk disease.

Experts say that by identifying this important [genetic marker](#) it means patients should be put on ALK inhibitors at the time of diagnosis with the hope of a cure.

Personalized treatment

Professor Deborah Tweddle, from the Newcastle University Centre for Cancer and Honorary Consultant at The Newcastle Hospitals NHS Foundation Trust, led the UK part of the study.

She said: "By identifying ALK as a genetic marker associated with the likelihood of a poorer outcome within the high-risk group of patients, it means we can treat these patients differently from the outset.

"Most excitingly there are new treatments that target the ALK protein itself, used in other cancers that can now be used for patients with high-risk neuroblastoma with ALK gene abnormalities.

"This research is an excellent example of personalized medicine. By treating those patients with an ALK genetic abnormality with an ALK inhibitor we are tailoring the treatment to the patients' individual tumor type.

"By combining an ALK inhibitor with the other treatments we currently give for high-risk neuroblastoma we hope to be able to cure more patients with this aggressive childhood [cancer](#)."

Neuroblastoma is a cancer of the nervous system that mainly affects children under five. It often begins in the [adrenal gland](#), but in around half of cases has spread throughout the body when it is diagnosed—in these high-risk cases survival is only approximately 50 percent.

Sadly, barely half of the children live beyond five years after diagnosis, despite treatment including high dose chemotherapy, surgery, radiotherapy and immunotherapy.

Several European countries took part in the study and the UK-arm of the trial has been based at Newcastle University with the test being carried out within the Newcastle Genetics Lab, supported by grants from Solving Kids Cancer and Neuroblastoma UK.

Study's findings

Experts carried out ALK testing on as many neuroblastomas as possible from patients treated on the high-risk trial and pooled this data with that from national genetic reference labs from 19 other countries.

The study showed that ALK mutations are present in about 14% of patients newly diagnosed with high-risk neuroblastoma, and nearly 4% have amplification of this gene.

More importantly, these abnormalities are associated with a lower rate of survival and they are therefore important prognostic markers that could be targeted by treatment.

Professor Tweddle added: "As part of the next European clinical trial for [high-risk neuroblastoma](#), we are going to treat all patients with an ALK abnormality found in their tumor with an ALK inhibitor in addition to standard treatment."

The breakthrough comes at the launch of the Newcastle University Cancer Fund, a new fundraising effort to inspire the next generation of cancer researchers to train and work at Newcastle for the benefit of people living with cancer locally as well as sending their research worldwide.

The Fund will work with cancer researchers at the very earliest stages of their studies and see them through to the point where they become established cancer researchers.

Patient case study

Happy schoolboy, Alexander Mohammed, is living life to the full as he enjoys fishing and outdoor activities with his family.

But five years ago, it was a very different story as the youngster, of Gosforth, Newcastle, was given the devastating diagnosis of neuroblastoma.

For almost two years Alexander had intensive treatment which included many aggressive cycles of chemotherapy at the Great North Children's Hospital in Newcastle.

The 10-year-old is now in remission and is monitored every six months under the care of Professor Deborah Tweddle.

Alexander's dad Raphael, a doctor, 43, said: "When Alexander was diagnosed with cancer it was such a shock and not something that we were expecting as he did not have the classic symptoms of neuroblastoma.

"It was difficult to watch him go through the intensive treatment, but he was so brave and to see how well he is doing now is fantastic as he has been in remission for four years.

"As a parent you do have at the back of your mind the worry that his condition could relapse and that's why it's great to see the research into neuroblastoma being done at Newcastle University.

"To hear of this major breakthrough into the treatment of the condition for children offers real hope of personalized treatment options in the future so that more youngsters lives can be saved."

More information: Angela Bellini et al, Frequency and Prognostic Impact of ALK Amplifications and Mutations in the European Neuroblastoma Study Group (SIOPEN) High-Risk Neuroblastoma Trial (HR-NBL1), *Journal of Clinical Oncology* (2021). [DOI: 10.1200/JCO.21.00086](https://doi.org/10.1200/JCO.21.00086)

Provided by Newcastle University

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