From molecules to medicine: cancer charity Neuroblastoma UK announces funding to accelerate rare childhood cancer research

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Neuroblastoma UK, a charity that solely funds research into neuroblastoma, a rare childhood cancer, is pleased to announce two research grant awards totalling £683,477, with a focus on improving treatment for children with advanced stages of the disease.

Around 100 children are diagnosed with neuroblastoma every year in the UK. Children with aggressive high-risk neuroblastoma are treated with incredibly intensive drugs and invasive procedures that can leave them with lifelong disabilities. And because they have an increased risk of relapse, the long term outcome of these children desperately needs to be improved. The grants will enable scientists to progress their research and develop safer, non-invasive and more effective treatment for children with the disease.

Professor Louis Chesler will receive a research grant of £469,093, which includes a donation of £15,000 from charity Friends of Rosie. Professor Chesler's research aims to develop blood-based biomarker tests for children with high-risk neuroblastoma, to help guide and monitor treatment.

Dr Mark Gaze will receive a research grant of £214,385 to develop a new type of targeted, radiotherapy using radioactive antibodies for children with high-risk neuroblastoma.

Tony Heddon, Chairman of Neuroblastoma UK said, "*We are delighted to award two large grants to help accelerate research into neuroblastoma. Traditionally, our charity has supported fundamental, early-phase research to help scientists develop a greater understanding of this complex and aggressive cancer.*

"As we move into our 40th year in 2022, it is important to us and our supporters that we fund research with the potential to benefit children quickly. A number of promising areas were highlighted which needed greater investment to help move them 'from bench to bedside' and into clinical trial. Thanks to voluntary donations and the generous support of Friends of Rosie, we are now able to support these two incredibly exciting research projects that we hope will lead to safer, more effective treatment for children with neuroblastoma."

Lisa Larkin, Founder and Trustee of Friends of Rosie (and Rosie's mum) said, "*For Friends of Rosie this particular research has extra resonance as Rosie, our charity's namesake and my daughter, had neuroblastoma. I remember so clearly being given the diagnosis and prognosis that she would die in the same sentence. There seemed to be a lack of priority for childhood cancer research and treatment. That's why we started Friends of Rosie - to pump prime vital new research to help children like Rosie. 30 years later we are delighted to collaborate with Neuroblastoma UK in this most exciting new project.*"

Postponed due to the coronavirus pandemic, Neuroblastoma UK launched their biennial research Grant Round for 2021 in April. The charity received seven research applications totalling £2.48million.

Professor Louis Chesler, Professor of Paediatric Cancer Biology at The Institute of Cancer Research said, "*To treat a child with neuroblastoma more effectively, we first need to understand how aggressive their cancer is or whether they are at greater risk of relapse. Currently the only way we can get detailed information about their tumour is from tissue biopsies, which are invasive and potentially dangerous. We do this in order to analyse tissue samples for molecular changes or 'biomarkers'. These biomarkers help us to diagnose the cancer and guide a child's treatment.*

"The medical technology now exists to detect multiple biomarkers in blood quickly and accurately, which could spare children from having to undergo painful tissue biopsies. But as yet, none of our standard clinical trials implement any blood-based testing, or molecularly guided drugs. This is a critical failure in modern clinical trial design that we wish to eliminate through our proposed work.

"Before we can bring these blood-based tests to trial, we need to formally evaluate the technology to determine its power and accuracy. We also need to cross-compare the various test types against each other to understand what they can and cannot measure. The research grant from Neuroblastoma UK and Friends of Rosie will enable our team to investigate blood samples and data from different biopsy

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techniques across three international research centres. Once we've completed our evaluation, we can then propose a less invasive method of diagnostic testing for children with this aggressive cancer.

“Our research team is hugely grateful to Neuroblastoma UK and their generous supporters for awarding us this grant. Thanks to donations from members of the public, we are able to continue our research work to move potential less invasive treatments from bench to bedside, a vital step in finding a cure for this rare childhood cancer."

Dr Mark Gaze, Consultant in Clinical Oncology at University College London Hospitals, said,

“When the cancer doesn't respond well to treatment, or comes back after earlier treatment in a child with high-risk neuroblastoma, one of the options is radiation delivered by a drug - we call that molecular radiotherapy. This is an established treatment, but it is not always effective, perhaps because some cells don't take up the radioactive drugs in current use well enough.

“Thanks to the grant from Neuroblastoma UK, our research will focus on developing a new radioactive drug which may be taken up better. This involves using an immunotherapy drug to deliver the radiation to cancer cells. Specifically, this immunotherapy will target GD2, an antibody which is highly expressed in neuroblastoma. We want to carry out a series of pre-clinical studies in which the anti-GD2 monoclonal - dinutuximab beta - is combined with novel radioisotopes to help deliver precise radiation doses. At the same time, our team will begin to prepare a clinical trial to see how effective radioactive dinutuximab beta is in treating the neuroblastoma.

“Our team is incredibly grateful for this opportunity to continue our research, something which wouldn’t be possible without the public’s generous donations to Neuroblastoma UK.”

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