BASE EDITING FOR GENTLE REPAIR OF FRAGILE DNA DISORDERS

Noah's Pink Balloon
LEUKAEMIA FUND

GREAT ORMOND STREET HOSPITAL CHARITY

BASE EDITING FOR GENTLE REPAIR OF FRAGILE DNA DISORDERS

OVERVIEW

This project is investigating a new DNA editing technique as a safer, more effective alternative to stem cell transplantation for children with genetic disorders.

THE NEED

Our DNA is a genetic material that carries instructions for our bodies to function properly. Sometimes, there can be mistakes or defects in the DNA that affect the way our cells repair themselves. These defects can lead to problems in a child's development, immune system, bone marrow, and increase their risk of getting cancer.

Doctors currently use a procedure called allogenic hematopoietic stem cell transplant (ASCT) - to treat these conditions. This involves transferring the stem cells from a healthy donor to the patient after chemotherapy or radiation to rebuild the immune system. However this procedure can come with risks.

Scientists are researching alternative treatments that involve editing the patient's own cells to fix the DNA defects by using a tool called CRISPR/Cas9. However, this tool can also cause new problems because it can create breaks in the DNA. Base editing is a technique that scientists can use to make precise changes to the DNA inside cells.

THE RESEARCH

Professor Waseem Qasim and his team at GOSH are investigating base editing - a potentially safer and more precise technique to change the abnormal DNA code back to normal. These new tools can make specific changes to the DNA without causing unwanted breaks. The techniques will be developed and tested in cells that have previously been collected from patients at GOSH or at the Great North Children's Hospital in Newcastle, UK. The most effective technique will then be tested on the collected blood and stem cells.

The team will then extensively test these novel techniques in the lab by thoroughly assessing the DNA code to ensure no unwanted effects have occurred and assess how the cells function. If the results show that DNA can be changed efficiently and safely, the team will aim to fix enough bone marrow cells in the hope to develop a kinder, more effective alternative to bone marrow transplantation which would then be tested in children as part of a clinical trial.

IMPACT

This project will help to better understand the utility of base editing techniques for genetic disorders, having implications for children with a myriad of conditions. The team are hopeful that the success of this work will enable the development of safer and more effective treatment as an alternative to ASCT.

THE DIFFERENCE YOU CAN MAKE

Research makes the untreatable, treatable. By supporting us to fund medical research at GOSH, you're helping to make the untreatable conditions of today, the treatable ones of tomorrow.

Your support of this medical research project can spark new discoveries and unlock life-changing breakthroughs that can significantly improve the lives of thousands of children in the UK and around the world.

Noah's Pink Balloon
LEUKAEMIA FUND

GREAT ORMOND STREET HOSPITAL CHARITY