



Gene Therapy Clinical Trial for patients with Mucopolysaccharidosis Type II (MPS II, Hunter Syndrome) opens in Manchester (United Kingdom).



What is MPS II and how is it treated?

MPS II is a genetic disorder that affects boys. Boys with MPS II don't have a functional enzyme known as iduronate-2-sulfatase (*IDS*) which is needed to break down long sugar chains in the body. When this enzyme is missing, these sugars build up to excess causing damage and stopping organs from working properly. Children with MPS II often have progressive symptoms such as developmental delay and physical problems.

The only approved treatment is enzyme replacement therapy where a regular infusion of the missing enzyme is given. But this treatment only helps some symptoms and cannot help problems in the brain.

What is Gene Therapy?

Gene therapy is a method by which a corrected gene is inserted into stem cells to treat diseases.

We are testing a gene therapy in children with MPS II. If you agree to take part, we will collect hematopoietic stem cells (HSC) from the blood and insert a working copy of the *IDS* gene into these cells in the laboratory. The modified HSCs are then infused back into the child where they can travel to the bone marrow. These cells will produce blood cells that can make the *IDS* enzyme and can carry it around the whole body, including the brain. This means the excess sugar molecules can be broken down and help cells to function better. We think this will reduce MPS II symptoms and may help to prevent damage to the brain.

How can I get involved?

We aim to test this gene therapy in 5 children with MPS II.

If your child has the severe form of MPS II and is aged between 3 months and 12 months, they may be eligible to join our study.

Screening, gene therapy and initial 2-year follow up will take around 28 months.

During this time, your child will need to visit Manchester, UK at regular intervals. Extended follow up of a minimum of 13 years is expected.



Please contact Professor Rob Wynn for further information on the trial
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