

New therapy could treat children with Hunter disease

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A team at the University of Manchester have developed a novel stem cell gene therapy approach to treat children with a devastating genetic disease. The approach is currently being developed for clinical trial in patients with the disease.

Hunter disease affects the bones, joints, hearts and lungs of affected children and in about two thirds of cases also affects the brain, leading to severe mental disabilities. It is a genetic disease inherited by affected boys, caused by the missing IDS gene.

Although there is an existing [enzyme replacement therapy](#) available currently on the NHS, it costs more than £150,000 per year per patient and doesn't penetrate to the brain.

Professor Brian Bigger's team at The University of Manchester developed a stem cell gene [therapy](#) approach that works by replacing the missing gene in the bone marrow of affected children. To make this more effective, they also added a tag to the IDS enzyme to allow it to pass into the brain.

The team demonstrated complete correction of both the bone, joint and brain disease in mice, recently published in the journal *EMBO Molecular Medicine*.

Prof Bigger said "We expected the stem cell gene therapy approach to deliver IDS enzyme to the brain, as we have shown previously for

another disease: Sanfilippo types A and B, but we were really surprised to discover how much better the tag made the therapy in the brain.

"It turns out that the tag didn't only improve enzyme uptake across the blood [brain](#) barrier, but also improved uptake of the [enzyme](#) into cells and it appeared to be more stable in the bloodstream – all improvements on current technology."

The team are part of the recently awarded InnovateUK Manchester Advanced Therapy Centre Hub (iMATCH), and aim to develop this therapy within this framework to take it to clinical trial.

A video describing the effects of the [disease](#) and potential of gene therapy can be seen here in Living Beyond Hope: A Short Documentary by Joshua Davies.

He added: "We are extremely grateful to our charity funders without whom this work would not have been possible, including the Isaac Foundation, the National MPS Society and the Newlife Foundation.

More information: Hélène FE Gleitz et al. Brain-targeted stem cell gene therapy corrects mucopolysaccharidosis type II via multiple mechanisms, *EMBO Molecular Medicine* (2018). [DOI: 10.15252/emmm.201708730](#)

Provided by University of Manchester

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