

Transplanting stem cells directly into brain could alter treatment of nervous system disorders

A therapeutic technique to transplant blood-forming (hematopoietic) stem cells directly into the brain could herald a revolution in our approach to treating central nervous system diseases and neurodegenerative disorders.

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The team's [findings](#) are groundbreaking because, until now, it was thought that HSCs — from a healthy, matched donor or a patient's own genetically-corrected cells — needed to be transplanted indirectly, through an intravenous line to the bloodstream.

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[I]n children with lysosomal storage disorders, caused by enzyme imbalances that result in a dangerous build-up of lipids, carbohydrates or other materials in the body's cells, time is of the essence to stop the disease's progression.

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"It can take up to a year for the genetically-engineered cell lineage to proliferate, spread and produce therapeutic effects in the brain — oftentimes, patients don't have the luxury of time to wait," [Researcher Alessandra] Biffi adds.

Biffi and her team wanted to find a faster — and more direct — way to transplant therapeutic HSCs into the brain.

In a mouse model of lysosomal storage disorders, Biffi's team transplanted HSCs — which they had genetically engineered to correct the enzyme imbalance — directly into the brain. They found the direct approach jumpstarted the therapeutic benefits much faster than intravenous infusion alone. They call their method, which infuses the cells into fluid-filled cavities in the brain called ventricles, "intracerebroventricular" delivery.

Read full, original post: [Routing gene therapy directly into brain](#)