

Realizing the potential of gene therapy for neurological disorders

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Promising findings from preclinical animal studies show the potential of gene therapy for treating incurable neurological disorders. In new research presented today, scientists successfully used gene therapy to slow the progression and improve symptoms of disorders such as amyotrophic lateral sclerosis and Parkinson's disease. The findings were presented at Neuroscience 2018, the annual meeting of the Society for Neuroscience.

Gene therapy typically employs an inactivated virus to carry new genetic cargo into cells, altering specific genes to treat or prevent a disease. Researchers might replace a mutated gene with a healthy copy of the gene, turn off a disease-causing gene, or add a new gene to the body to help fight a disease.

Although [gene therapy](#) is a promising treatment option for a limited number of conditions, including certain cancers, the technique is still experimental for most diseases, with ongoing research to ensure they will be safe and effective in human patients. Animal studies are a key part of the process by which an [experimental gene therapy](#) treatment goes to clinical trial.

Today's new findings show that:

- A new technique allows gene therapy to be delivered to the entire spinal cord in mice (M. Bravo Hernandez, abstract 208.10).
- Gene therapy safely and effectively extends life and improves motor function in a [mouse model](#) of ALS (Gretchen Thomsen, abstract 208.16).
- Gene therapy slows the progression of neuronal loss in a mouse model of inherited Parkinson's disease (Jose L. Lanciego, abstract 292.01).
- Gene therapy shows promise in a mouse model of Batten disease, a childhood neurodegenerative disease (Shibi Likhite, abstract 355.01).

"Gene therapy holds the promise to transform the lives of patients with incurable neurological diseases," said press conference moderator Fredric Manfredsson, Ph.D., of Michigan State University. "The research presented today represents important and exciting steps toward being able to prevent and treat disorders that currently have no cure, such as Parkinson's disease and Alzheimer's disease."

Provided by Society for Neuroscience

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