Regenerative Medicine for Drug Resistant Epilepsy (DRE)

Neurosurgeon, Jonathon J. Parker, is the lead investigator of the first-in-human clinical trial at Mayo Clinic studying the use of implanted specialized inhibitory brain cells as a potential reparative treatment for DRE. The clinical trial is underway at Mayo Clinic in Arizona.

“We use a very minimally invasive technique where we inject the inhibitory cells through a pencil eraser-sized incision in the back of the head. Our hope is that, over time, these cells become part of the brain and help repair the neural circuitry, and reduce or prevent seizures without the side effects,” says Dr. Parker.

Read more at https://newsnetwork.mayoclinic.org/discussion/mayo-clinic-researchers-lead-transformative-shift-toward-neurorestorative-treatment-strategies-for-most-severe-forms-of-epilepsy/

Website accessed on May 8, 2025.

Deep Brain Stimulation (DBS) for DRE

Researchers at Mayo Clinic in Minnesota have developed an innovative DBS platform that was used not only to reduce seizures, but also improve memory and sleep – two common challenges for patients with epilepsy.

Researchers found that low-frequency DBS not only reduced seizures, but also improved sleep and memory. “Using an implanted investigational device, the team continuously

monitored brain activity with AI-driven seizure and sleep tracking," says Gregory Worrell, M.D., Ph.D., Mayo clinic neurologist and co-lead author of the study.

Read more at https://newsnetwork.mayoclinic.org/discussion/new-study-in-brain-communications-finds-personalized-deep-brain-stimulation-shows-promise-for-drug-resistant-epilepsy/

Website accessed on May 8, 2025.

Stem-Cell Transplant Treatment

Jonathan Nemeth's epilepsy is so severe that muscle contractions from seizures broke his spine twice. Effects of the neurological disorder also postponed his typical life milestones, such as getting a driver's license.

Despite those challenges, the 22-year-old is cautiously optimistic. He has been seizure-free since undergoing a pioneering operation in February that involved transplanting stem cells into the area of his brain causing his seizures. The experimental treatment is part of the first in-human clinical trial investigating the safety and efficacy of lab-grown interneuron cells to treat seizures in deep areas of the temporal lobes on both sides of the brain.

"This is a first step in treating the underlying biology of this type of epilepsy," said Peter Warnke, MD, the trial's local principal investigator.

Read more at https://www.uchicagomedicine.org/forefront/neurosciences-articles/first-of-its-kind-stem-cell-transplant-treatment-targets-drug-resistant-focal-epilepsy

Website accessed on May 9, 2025

Access to Surgery

Recent research has shown that pediatric epilepsy surgery is performed in a small proportion of eligible pediatric patients with drug resistant epilepsy (DRE), with estimates ranging from 1% to 11%. In a review, researchers identified potential influences such as family perceptions of surgery, provider familiarity with treatment pathways, and healthcare system-level

disparities. Authors noted that these findings suggest that additional efforts across patient, provider, and system levels may help address barriers to access to epilepsy surgery for pediatric patients.

Read more at https://www.neurologylive.com/view/improving-access-surgical-evaluation-drug-resistant-epilepsy-satyanarayana-gedela

Website accessed on May 9, 2025.

Common Genetic Variants Linked to DRE

Certain common genetic changes might make some people with focal epilepsy less responsive to seizure medications. Antiseizure medication is usually prescribed for people with the condition; however, for one in three people with epilepsy, current antiseizure medications are ineffective.

These findings are particularly important as these genetic signatures can be determined at the onset of epilepsy, rather than after several antiseizure medications have been tried without success. This could eventually help to predict which individuals with epilepsy are likely to develop drug resistance and avoid unnecessary exposure to ineffective medications and their associated side effects.

Read more at https://www.cureepilepsy.org/news/common-genetic-variants-linked-to-drug-resistant-epilepsy/?utm_source=CURE+Epilepsy+Email+List&utm_campaign=adc6a4de3e-ERN+May+2025&utm_medium=email&utm_term=0_-adc6a4de3e-294568577

Website accessed May 21, 2025.

Biomarker Helps Presurgical Evaluations

In a recent study, researchers on the Cook Children's Neurology team examined the temporal relationship between electrographic features on an EEG known as spikes, ripples, and fast ripples, and assessed the ability of these EEG features (and their combination) to indicate the brain area that causes seizures; better isolation and targeting of this brain area for resective surgery could improve surgical outcomes.

Read more at https://www.cureepilepsy.org/news/novel-biomarker-helps-presurgical-evaluations-in-children-with-drug-resistant-epilepsy/?utm_source=CURE+Epilepsy+Email+List&utm_campaign=adc6a4de3e-ERN+May+2025&utm_medium=email&utm_term=0_-adc6a4de3e-294568577

Website accessed on May 22, 2025.

Split Gene Therapy Delivers Promise in Mice Modeling Dravet Syndrome

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A potential new gene therapy for Dravet syndrome increases survival and prevents seizures, according to initial tests in mice. In a recent study, researchers engineered two viruses, each carrying a portion of a therapeutic version of the SCN1A gene and selectively targeted them to interneurons. These cells then translated the two halves of the gene and fused them to form the complete protein.

Administering the dual virus into the animals completely prevented premature death. When the researchers increased the body temperature of treated mice, they all appeared to remain healthy, whereas more than half of the untreated mice had seizures. The dual virus approach also reduced spontaneous seizures.

Read more at https://www.cureepilepsy.org/news/split-gene-therapy-delivers-promise-in-mice-modeling-dravet-syndrome/?utm_source=CURE+Epilepsy+Email+List&utm_campaign=adc6a4de3e-ERN+May+2025&utm_medium=email&utm_term=0_-adc6a4de3e-294568577

Website accessed on May 22, 2025.

Researchers Chart History of Patients with SCN8A-Related Disorders

Researchers have completed a comprehensive natural history study of SCN8A-related disorders. Variants in the SCN8A gene cause a spectrum of neurological conditions, including epilepsy, ranging in severity from mild to severe seizures, developmental delays, autism spectrum disorder and movement disorders.

An understanding of the genotype-phenotype correlations will be critical when designing outcome measures and selecting participants for future clinical trials.

Read more at https://www.cureepilepsy.org/news/childrens-hospital-of-philadelphia-researchers-chart-natural-history-of-patients-with-scn8a-related-disorders/?utm_source=CURE+Epilepsy+Email+List&utm_campaign=adc6a4de3e-ERN+May+2025&utm_medium=email&utm_term=0_-adc6a4de3e-294568577

Website accessed on May 22, 2025.