

# Testing a one-time treatment to relieve Parkinson's symptoms

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After decades of research into the causes and treatment of Parkinson's disease, UC Irvine Health neurologist Dr. Claire Henchcliffe is hopeful that a new cell therapy can finally bring meaningful relief to patients

with the progressive neurodegenerative movement disorder.

A national expert on Parkinson's disease, she is one of a small group of U.S. researchers conducting a first-in-human clinical trial of transplanted [stem cells](#) engineered to replace dopamine-producing neurons that are destroyed by the debilitating and incurable condition.

As the brain loses its ability to produce the potent neurotransmitting chemical, that leads to the tremors, stiffness, slowness and lack of coordination seen in Parkinson's patients.

The next-generation stem cell treatment, MSK-DA01, which restored the brain's ability to produce dopamine in animal studies, could have [profound implications](#) for the nearly 1 million Americans and 10 million people worldwide living with Parkinson's, says Henchcliffe, chair of the UCI School of Medicine's Department of Neurology and a principal investigator of the groundbreaking trial.

"The big advance here is being able to produce what looks like a one-size-fits-all, single treatment that could potentially provide lifelong relief from Parkinson's symptoms," she says. "Pre-clinical work has shown that these transplanted dopamine progenitor [cells](#), taken from [human embryonic stem cells](#), have improved movement and coordination."

### **Limits of medications**

Current treatments for Parkinson's patients are mainly focused on drugs that replace dopamine or dopamine-like substitutes. These medications provide relief for movement-related symptoms but usually for only a limited time.

"As the disease progresses, the medications become less effective and people end up tied to the pillbox," Henchcliffe says. "For people with

advanced Parkinson's, the medicines don't last long enough."

The new treatment being tested in patients with advanced Parkinson's involves embryonic stem cells transformed into dopamine-producing neurons that are surgically transplanted into the putamen, the area of the mid brain that is no longer producing dopamine.

After the surgery, trial participants receive immunosuppression treatment with intravenous and oral steroids for a year to help establish the transplanted nerve cells.

### **Testing safety, effectiveness**

Demonstrating the treatment's safety and efficacy are the primary goals of the trial, which is sponsored by BlueRock Therapeutics, a developer of engineered cell therapies to reverse disease based in Cambridge, Mass.

"Although we got excellent safety and tolerability data from animal studies, it has never been tested in humans," says Henchcliffe, who was part of the group at Memorial Sloan Kettering Cancer Center and Weill Cornell Medical Center that worked on developing the phase 1 clinical trial before she joined the UCI School of Medicine in 2020.

"A first-in-human study is always about making sure that a treatment is safe and does not cause unexpected side effects."

The transplantation takes place at Memorial Sloan Kettering Cancer Center in New York City. UCI Health trial participants are then followed by Henchcliffe and the Parkinson's Disease and Movement Disorders Program team in Irvine.

Over the next two years, researchers will study whether the implanted

cells survive and if they improve trial participants' motor functions.

## Improved technologies

Rapid advances in stem cell technology in recent years have made this clinical trial possible. Earlier clinical trials to restore dopamine function with cell transplantation showed promising but variable results, which she attributes to limitations of earlier stem cell sources.

"We've figured out how to make [embryonic stem cells](#) that can be grown in the laboratory in almost unlimited quantities," says Henchcliffe, who began her Parkinson's research 25 years ago.

"Now researchers, including the people I worked with at Memorial Sloan Kettering, have found a way to differentiate those cells into dopamine-producing neurons. With new technologies coming up, we foresee some really accelerated advances for patients."

The longtime movement disorders physician and scientist is thrilled that this cell therapy trial, the culmination of decades of research efforts, is available to UCI Health patients.

"Patients who are excited about this trial see it as something that looks more like a cure," she says. "Something that can restore their abilities, something that gets more to the fundamentals of Parkinson's disease, rather than a [treatment](#) disguising the symptoms."

Provided by University of California, Irvine

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