

Promising respiratory drug focus of new clinical trial for Parkinson's disease

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A medication approved to treat various respiratory diseases and that has demonstrated neuroprotective effects in preclinical studies is the focus of a new clinical trial for Parkinson's disease.

The trial is the latest to be launched as part of the Linked Clinical Trials (LCT) initiative, a program spearheaded by UK research charity The Cure Parkinson's Trust in collaboration with Van Andel Research Institute (VARI), which is based in Grand Rapids, Michigan, USA.

"With this trial, we're moving beyond treating Parkinson's symptoms. We want to actually slow or stop disease progression," said Patrik Brundin, M.D., Ph.D., head of LCT's international scientific committee and director of VARI's Center for Neurodegenerative Science. "This drug—ambroxol—has performed exceptionally well in [preclinical studies](#) for Parkinson's and is already approved to treat other conditions."

By focusing on existing medications that have already passed the rigorous drug approval process and shown promise in preclinical laboratory studies for treating Parkinson's, LCT aims to significantly reduce the time and cost required to bring new therapies to people with Parkinson's.

"Linked Clinical Trials has real momentum and provides us with the prospect of having new, potentially breakthrough treatments being accelerated into the clinic within a relatively short period," said [Tom](#)

[Isaacs, president and co-founder of The Cure Parkinson's Trust](#). "This new trial provides real hope that we are on the cusp of something truly life-altering—new therapies that show promise to improve the quality of life for the seven to 10 million people worldwide who have Parkinson's.

From respiratory remedy to neuroprotection

The trial is based at Royal Free Hospital in London and focuses on ambroxol, which is typically used to treat respiratory conditions. Earlier work conducted by the trial's principal investigator Anthony Schapira, M.D., D.Sc., demonstrated that ambroxol improves the function of a protein that plays a key role in cellular "trash removal" processes. Recent evidence suggests that impairment of these processes are linked to neurodegenerative disease onset and progression. Additionally, mutations in the gene that encode this protein are considered to be the greatest genetic risk factor for developing Parkinson's.

"Our preclinical work suggests ambroxol may be an effective Parkinson's treatment thanks to its ability to correct a dysfunctional protein that is prevalent in people carrying a genetic mutation associated with inherited Parkinson's cases," Schapira said. "What is particularly interesting is the potential for ambroxol also to benefit Parkinson's patients without these genetic mutations."

A complex problem

There have been few major therapeutic breakthroughs for Parkinson's in the last 50 years with the exception of levodopa, the current gold standard for drug treatment, and deep brain stimulation, a surgical option. Although these therapies may significantly improve quality of life, they do not slow or stop the progressive brain cell death that is the disease's hallmark. LCT's scientific committee, which comprises leading Parkinson's experts and advocates from around the world, is charged

with investigating additional compounds that not only treat symptoms but that may also stop [disease progression](#).

In addition to the ambroxol trial, LCT also supports trials to explore the cholesterol drug simvastatin and the diabetes drug exenatide as potentially disease modifying treatments in Parkinson's. In the coming months, additional trials focused on other medications, including other diabetes treatments, drugs to remove iron build-up in the brain, and treatments to address mitochondrial function, are expected to launch.

Provided by Van Andel Research Institute

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