FDA clears investigational drug for ALS gene therapy trials

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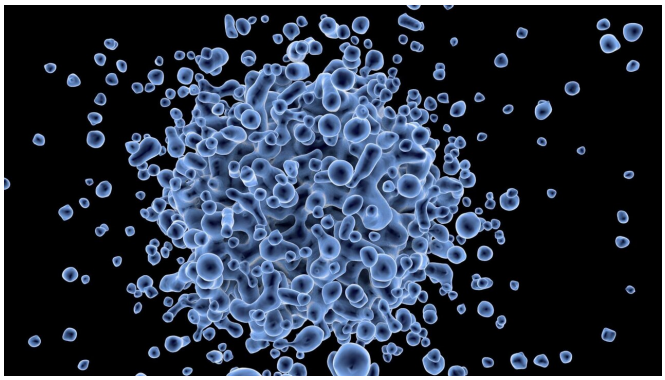

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Apic Bio, a gene therapy company developing treatment options for patients with rare genetic diseases and co-founded by UMass Medical School's Robert H. Brown Jr., DPhil, MD, announced that the FDA has cleared its investigational new drug (IND) application for APB-102, a gene therapy candidate designed to treat a common cause of familial amyotrophic lateral sclerosis.

"The clinical development of APB-102 is rooted in nearly 30 years of gene therapy research demonstrating the link between the SOD1 gene mutation and ALS and the strong potential of AAV-delivered SOD1 targeting miRNA to slow down or reverse the progression of ALS in patients with SOD1 mutations," said Dr. Brown, the Leo P. and Theresa M. LaChance Chair in Medical Research, professor of neurology and scientific co-founder of Apic Bio.

Brown was a lead member of the team that discovered SOD1 as the first genetic mutation linked to ALS.

"Despite SOD1 gene mutations being well understood as a cause of genetic ALS for decades, we don't yet have an approved treatment option that targets the disorder at the source," said Brown. "I am pleased to see APB-102 progress as Apic Bio aims to develop a meaningful, long-term disease-modifying gene therapy option for patients."

APB-102 is designed to slow or reverse progression of SOD1 ALS through a recombinant adeno-associated virus (AAV) capsid and micro ribonucleic acid (miRNA) vector construct, which has been shown in preclinical proof of concept studies to suppress activity of the mutated SOD1 gene.

The Phase I/II clinical trial will be initiated in late 2021 or early 2022 as a multi-center, three-part study to evaluate the safety, tolerability and efficacy of APB-102 in patients with SOD1 ALS mutations.

ALS is a fatal neurodegenerative disorder characterized by loss of motor neurons, leading to muscle weakness and eventual paralysis. Most patients face mortality within five years of disease onset due to respiratory failure. ALS can be caused by multiple genetic mutations and can be sporadic or familial. Approximately 10 percent of ALS cases have a known genetic driver; of those, approximately 20 percent are linked to a mutation in the SOD1 gene that codes for the enzyme superoxide dismutase 1. Current approved ALS treatments only delay disease progression without addressing the underlying genetic causes of the disease.

Provided by University of Massachusetts Medical School