

ALS Research Has a Bright Future

Amyotrophic lateral sclerosis (ALS) research truly has a pipeline of promise, with progress being made on three fronts: improved trial methods, new drug formulations, and a new therapy approved by the US Food and Drug Administration (FDA).

On April 25, the FDA [granted accelerated approval](#) for Biogen's drug tofersen (Qalsody) to treat patients with ALS associated with a mutation in the superoxide dismutase 1 (SOD1) gene (SOD1-ALS). Qalsody is an antisense oligonucleotide that targets SOD1 mRNA to reduce the synthesis of SOD1 protein.

"It is really exciting to see tofersen approved for patients with familial ALS due to SOD1 mutation," says MDA Care Center Director Senda Ajroud-Driss, MD, a neurologist and Associate Professor of Neurology at the Northwestern University Feinberg School of Medicine and Director of the Lois Insolia ALS Clinic and the Les Turner ALS Foundation. "This is a very important step in our quest for a world free of ALS."

Researchers also see hope in ongoing clinical trials.

“We now have an innovative way to test new therapies in ALS called the platform trial,” Dr. Ajroud-Driss explains. “We can test multiple drugs at the same time since there is significant interest from many pharmaceutical companies in ALS.”

Platform trials are a type of clinical trial where multiple interventions can be evaluated simultaneously against a common control group within a single master protocol. (Learn more about platform trials and how to interpret results [here](#).) These trials typically cost less and take less time than traditional clinical trials.

According to Massachusetts General Hospital, where the [HEALEY ALS Platform Trial](#) was started, this model reduces research costs by 30% and increases patient participation by 67%.

So far, in the HEALEY ALS Platform Trial, which has taken place over the last three years, two drugs were negative, and two drugs had a signal to go for further testing. The trial continues to add other drugs, and enrollment will continue as more investigational therapeutics enter the mix.

There is enthusiasm in the research community for new and reformulated ALS drugs.

Radicava, which has been shown to slow functional decline in people with ALS, has been reformulated into a liquid form (Radicava ORS) that can be taken by mouth or through a feeding tube.

Dr. Ajroud-Driss pointed to a new treatment called sodium phenylbutyrate/taurursodiol ([RELYVRIO](#)). “The combination of two drugs is supposed to slow down the progression of the disease,” she says.