BIOPHARMADIVE

BRIEF

# Biohaven adds to a long list of Alzheimer's drug failures

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By Jacob Bell

## Dive Brief:

- Alzheimer's drug research notched yet another setback Monday, as a late-stage clinical trial found that an experimental medicine from Biohaven Pharmaceuticals did not significantly impact brain function in patients with mild to moderate disease.
- Biohaven said that after 48 weeks of treatment, its drug, troriluzole, was statistically no different than placebo, as measured by two scales used to evaluate Alzheimer's disease and dementia. The company claims there were some potentially promising results among just the patients with mild disease, but further analysis is needed to determine whether troriluzole warrants additional study in early Alzheimer's.
- Full results from the trial will be presented at an upcoming scientific meeting, according to Biohaven. In the meantime, the company plans to amend an ongoing extension study so that mild Alzheimer's patients can continue receiving troriluzole and researchers can gather additional clinical and biomarker data.

## Dive Insight:

Alzheimer's is one of the world's most pressing health crises. The disease affects millions and is a leading cause of death for older people. It's also an enormous burden on healthcare systems; in the

U.S. alone, treating Alzheimer's cost an estimated $305 billion last year.

While there are treatments for the symptoms of Alzheimer's, such as memory and attention loss, so far no drug has been approved to alter the course of the disease. There's a chance a first-of-its-kind approval could come soon, as the Food and Drug Administration is currently reviewing an antibody therapy from Biogen, with a decision expected by early March.

Biogen claims that clinical studies of the therapy, known as aducanumab, offer enough evidence to say it slows cognitive decline in patients with early Alzheimer's. But in November, advisors to the FDA strongly rebuked aducanumab's case for approval, which then left its future in doubt. Though the FDA isn't required to follow advisors' recommendations, it typically does.

Outside of aducanumab, the track record for these so-called disease-modifying drugs has been almost entirely negative. A laundry list of experimental medicines — from AstraZeneca, Biogen, Eli Lilly, Johnson & Johnson, Merck & Co., Roche and many others — have suffered major setbacks. Now, troriluzole joins them.

"This study was well-conducted but unfortunately it is clear from this preliminary analysis that troriluzole is not efficacious as a symptomatic treatment in a mixed population of patients," Vlad Coric, Biohaven's CEO, said in a statement Monday.

Biohaven did note that, at the end of the study period, the mild Alzheimer's patients treated with troriluzole showed a nonsignificant numerical difference on one of those cognitive scales, as well as another, secondary assessment that looked at the size of the hippocampus, a part of the brain integral to memory. The company said these numerical differences could suggest "a

potential biologic effect of troriluzole in patients," but acknowledged that further analysis is needed.

Investors don't appear to be holding out much hope, however. Biohaven shares were down around 10% at market's open Monday, to trade at $76.35 apiece.

Troriluzole is a prodrug of riluzole, the active ingredient in a couple of approved drugs used to treat ALS.