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Democracy Dies in Darkness

In surprise turnaround, new analysis finds an Alzheimer's treatment may work

By **Tara Bahrampour**

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Seven months after clinical trials for a promising Alzheimer's drug were halted and the treatment was declared a failure, a new analysis suggests it was actually effective, and the company that makes it plans to move forward in securing federal approval.

The astonishing reversal on aducanumab, an antibody therapy that targets a protein called amyloid beta that builds up in the brain, comes after new data from the discontinued studies showed that at high doses the drug reduced cognitive decline in patients with early Alzheimer's.

"It could be a game-changer for the field," said Rebecca Edelmayer, director of scientific engagement at the Alzheimer's Association. "It could be one of the first disease-modifying therapies approved for Alzheimer's disease."

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An estimated 5.7 million Americans 65 and over have Alzheimer's, and the Alzheimer's Association expects that number to mushroom to nearly 14 million by 2050 in the absence of new treatments. A handful of drugs approved by the Food and Drug Administration can alleviate some symptoms, but in the past 16 years no new drugs have been approved for the disease.

That may now change.

The drugmaker, Biogen, said that patients receiving aducanumab experienced "significant benefits on measures of cognition and function such as memory, orientation and language." They also saw benefits in activities of daily living, including conducting personal finances, performing household chores and traveling independently outside the home.

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Biogen said it plans to pursue regulatory approval for aducanumab in the United

States and continue discussion on it with regulatory agencies internationally. The announcement sent the company's stock price soaring Tuesday morning.

The company had stopped the trials in March after an independent monitoring board said the drug offered little hope of success, sending waves of disappointment through the scientific community after earlier trials had looked promising.

"The field was really pinning its hopes that aducanumab would be positive, would show results," said Ronald Petersen, director of the Mayo Clinic Alzheimer's Disease Research Center and the Mayo Clinic Study of Aging, who consulted with Biogen on the drug but was not involved in the studies. "It did call into question whether attacking amyloid at all was a viable strategy."

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But analysis that included data gathered in the months after the monitoring board's analysis appeared to show that one of the trials had in fact met its primary goal, though the other had not.

The FDA said on Monday that Biogen could file for approval for the drug; the company expects to do so in early 2020. Biogen said it aims to offer it to patients who were previously enrolled in those studies.

If approved, aducanumab would become the first therapy to reduce the clinical

decline of Alzheimer's disease. It would also bolster the theory that treatments that remove or reduce amyloid beta, which creates plaques associated with the disease, are an effective approach.

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Other therapies now in the clinical trial pipeline include those that address inflammation, the immune system, blood vessels and synaptic cell health. Experts say an effective treatment for the disease is likely to involve a combination of several therapies.

"We need to continue these different approaches because we think that a treatment is potentially going to be complex," Edelmayer said.

While Petersen called the news "a bright light" after "so many failures in the field," he cautioned that it is not clear the FDA will approve the drug, particularly in light of the fact that one of the trials failed and traditionally two successful trials have been required for approval.

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In the failed trial, fewer people had received the high dose of the drug for a sufficient period of time, he said, adding that people in a subset of that trial who had received higher doses had seen improvement.

"The challenge, of course, is to convince the FDA that therefore those are believable," he said.

As the news boosted the stocks of Biogen and other companies with amyloid-targeting therapies, Baird analyst Brian Skorney warned in a note to clients that given the failure of such therapies in the past, the company faces an "uphill climb" in making the case to regulators that the positive results were "nothing more than random chance."

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