

# FDA panel reviews 1st new Alzheimer's drug in 2 decades

November 6 2020, by Marilyn Marchione and Matthew Perrone

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In this Dec. 12, 2019 photo provided by Biogen, a scientist works on Alzheimer's disease research in a laboratory at the drugmaker's headquarters in Cambridge, Mass. On Friday, Nov. 6, 2020, a panel of outside experts will meet to advise the Food and Drug Administration on the drug, called aducanumab from Biogen Inc. and Japan's Eisai Co. The drug does not cure or reverse Alzheimer's; the claim is that it modestly slows the rate of decline. (David A. White/Biogen via AP)

One of the biggest drug decisions in decades is looming as U.S. regulators consider whether to approve the first medicine that's claimed to slow mental decline from Alzheimer's disease, the most common form of dementia.

A panel of outside experts meets Friday to advise the Food and Drug Administration on aducanumab, a [drug](#) from Cambridge, Massachusetts-based Biogen Inc. and Japan's Eisai Co. FDA doesn't always follow the panel's advice but usually does and has until March to decide.

The drug does not cure or reverse Alzheimer's; the claim is that it modestly slows the rate of decline.

The evidence is murky: The companies stopped two studies last year when the drug didn't seem to work, then did an about-face and said additional results suggest it was effective in one study at a high dose. Results still have not been published.

An FDA staff report released Wednesday gave a generally glowing view, saying the positive study might be "exceptionally persuasive." But an FDA statistician noted flaws and inconsistencies in the results and potential safety issues.

The drug is expected to be very expensive and "could bankrupt our health care system" while giving patients false hope, the consumer group Public Citizen warned.

More than 5 million people in the United States and many more worldwide have Alzheimer's. Current drugs only temporarily ease symptoms and the last one was approved nearly two decades ago.

The FDA evaluation focuses on safety and effectiveness. But advocates for approval, including the Alzheimer's Association, are pushing to make

need part of the decision.

"There is a dire and drastic need" to help people impacted "by the crushing realities" of the disease, the association wrote to the FDA. The association got \$450,000 from Biogen and Eisai last year, less than 1% of its total revenue.

Others say the need for a drug has no bearing on whether it works or is safe.

"This is a difficult decision. I think it's going to be contentious no matter how it falls out," said Dr. Howard Fillit, chief science officer of the Alzheimer's Drug Discovery Foundation, who consults for Biogen.

## ABOUT THE DRUG

Aducanumab (pronounced "add-yoo-CAN-yoo-mab") aims to help clear harmful clumps of a protein called beta-amyloid from the brain. Other experimental drugs have done that but it made no difference in patients' ability to think, care for themselves or live independently.

It's a biotech medicine made from living cells, and such drugs are very expensive. No price estimate has been announced for the drug, which is given through an IV once a month. Fillit notes that Biogen drugs for other diseases cost \$3,500 a month, plus fees for each infusion.

If aducanumab is approved, it's expected to be covered by Medicare, the government plan for seniors. The FDA and Medicare are barred from considering cost when reviewing a new drug or treatment.

Even qualifying for the drug could be expensive. It's only been tested in people with mild dementia from Alzheimer's or a less severe condition called mild cognitive impairment. To verify a diagnosis has required

brain scans that cost \$5,000 or more. Insurers including Medicare don't cover the scans because their benefits are unclear, but that could change if a scan becomes a gateway to treatment.

## THE EVIDENCE

Historically, the FDA often required two studies showing safety and effectiveness, but in recent years has relaxed that standard.

Each of the two aducanumab studies enrolled about 1,650 people and were stopped roughly halfway through when it seemed the drug wasn't working. Biogen says that later results show one study was positive at the highest dose; the second study was clearly negative. The company says an analysis from both studies on people who got the highest dose for the longest time shows benefit.

But there are many questions about the validity of such analyses. Another complication: the studies were changed after they were underway to let some people get a higher dose. And the placebo group in the positive study worsened more than the one in the negative study did, which could help explain why aducanumab appeared better by comparison in that one.

The FDA review largely dismissed safety concerns, including swelling in the brain that occurred in as many as one-third of patients, often leading to discontinuation of the drug.

The FDA should require a third study to test the drug in ideal conditions and get a clear answer, said the Mayo Clinic's Dr. David Knopman. He's on the FDA [advisory panel](#) but won't vote on aducanumab because he helped lead one study. He and other doctors [published a journal report](#) earlier this week arguing against approval.

## WHAT IT WOULD MEAN FOR PATIENTS

Any benefit from the drug "is relatively small," said Dr. Eliezer Masliah, neuroscience chief at the U.S. National Institute on Aging.

In the positive study, the drug modestly slowed the rate of mental decline—a difference of only 0.39 on an 18-point score of thinking skills. How much that means in terms of being able to live independently, recognize family members or remember things is unclear.

Drugs that remove amyloid may have to be combined with medicines that do other things in the brain, and used early enough before damage occurs, to do much good, Masliah said.

If the drug is approved, the American Academy of Neurology urged the FDA to not make it a broad authorization, which could expose many patients to a medicine that might harm rather than help, and could "overwhelm the health care system."

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Citation: FDA panel reviews 1st new Alzheimer's drug in 2 decades (2020, November 6)  
retrieved 19 September 2024 from  
<https://medicalxpress.com/news/2020-11-fda-panel-1st-alzheimer-drug.html>

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