There's a new Alzheimer's drug, so what's the problem? A doctor explains.

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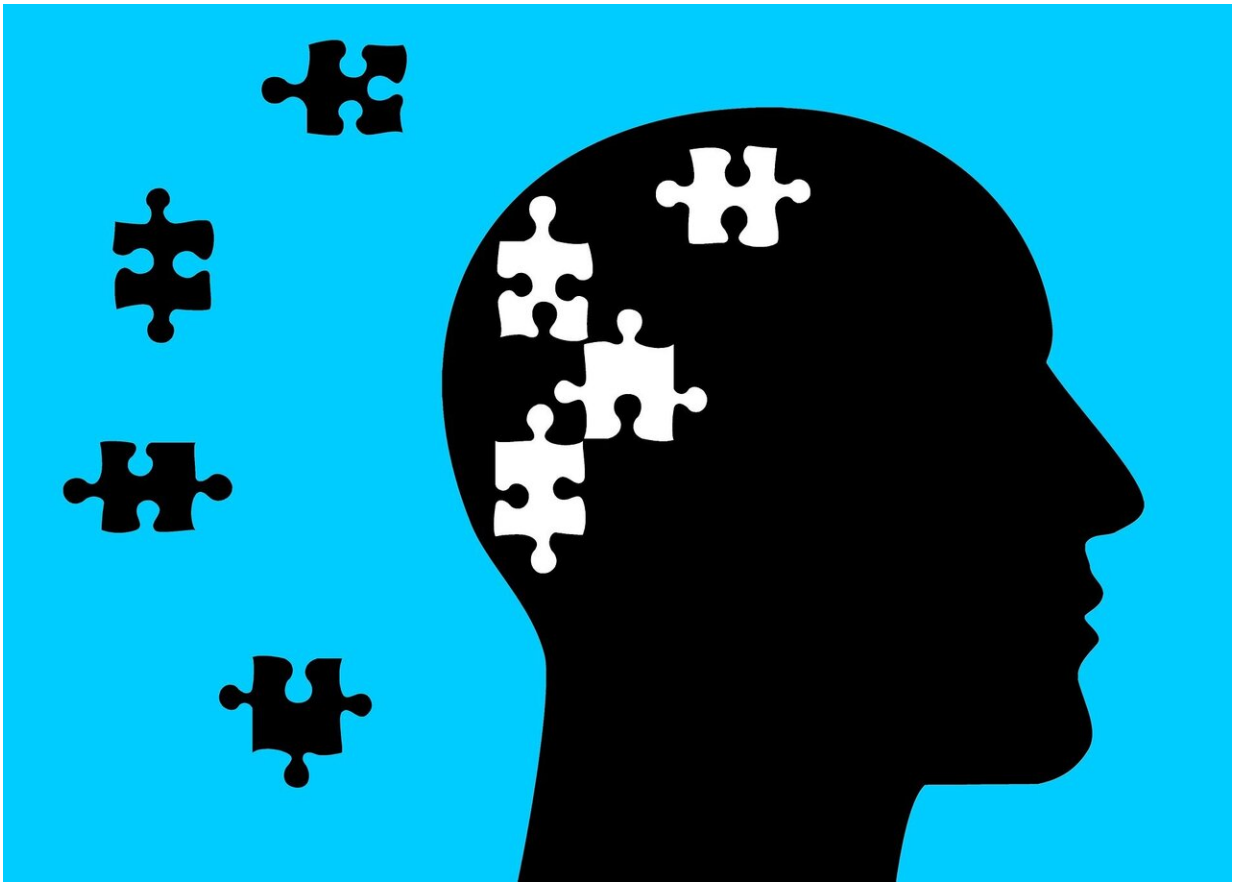

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After years of disappointing studies of potential treatments for the more than 6 million people living with Alzheimer's disease, the U.S. Food and

Drug Administration recently approved a new drug called aducanumab that is sure to garner significant interest.

The drug, however, followed an unusual regulatory path and did not convince a key advisory panel that it works.

Dr. G. Caleb Alexander, an internist and a professor of epidemiology and medicine in the Johns Hopkins Bloomberg School of Public Health, served on that committee and spoke with The Baltimore Sun about the new drug.

So why all the buzz about this drug?

There is no cure for Alzheimer's, a form of dementia characterized by memory loss, confusion and eventual death. No new drug has been approved since 2003. Alexander said there is need and demand for new treatments.

Advocacy groups had pushed for approval of this therapy that could hold off the symptoms, though not stop progression of the disease.

Harry Johns, the president and CEO of the Alzheimer's Association, called aducanumab's approval a victory for people living with Alzheimer's and their families, and said it will give patients "more time to live better." But rather than an endpoint, he said, the approval is "about reinvigorating scientists and companies in the fight against this scourge of a disease."

What did the studies show?

Biogen Inc., a Cambridge, Massachusetts-based biotech company, and the Japanese pharmaceutical company Eisai Co. developed the drug

named Aduhelm and tested it in three large clinical trials in people with early Alzheimer's disease.

Studies were halted in 2019 after showing no benefits but revived after the companies took another look at the data from one of the studies. They found it reduced a sticky protein in the brain called beta-amyloid.

Alexander said that when that protein accumulates, that plaque is the chief suspect in brain cell death and deterioration of patients.

This is the first drug shown to reduce the plaque.

"We believe this first-in-class medicine will transform the treatment of people living with Alzheimer's disease and spark continuous innovation in the years to come," Biogen CEO Michel Vounatsos said in a statement.

How did the drug win approval from the FDA?

The FDA gave the drug accelerated approval, a process reserved for treatments aimed at serious or life-threatening illness. It was based on the reasonable likelihood that it would produce benefits for patients, according to the regulators, who are requiring the companies to continue studying the drug for years.

"Currently available therapies only treat symptoms of the disease; this treatment option is the first therapy to target and affect the underlying disease process of Alzheimer's," said Dr. Patrizia Cavazzoni, director of the FDA's Center for Drug Evaluation and Research, in a statement.

She said approval would bring a therapy to patients faster while "spurring more research and innovation."

What's unusual about this process?

Alexander said several things were unusual, from resurrecting the drug after failed trials to the FDA's approval based on data from one study with what he called "murky" evidence that it reduces symptoms.

The FDA's own advisory panel recommended against approval last fall.

"There is an incredible amount of unmet need in a real sense, a palpable urgency for patients and their loved ones who may be experiencing the disease firsthand," he said.

"But there is an enormous amount of uncertainty if the product works," he said. "Ultimately the FDA decided to allow approval of the product not on the basis of its demonstrative effectiveness in improving symptoms of Alzheimer's, but on the ability to lower the levels of beta-amyloid."

What happens now?

The companies can begin shipping the drug. Typically insurers will cover a treatment if it's FDA-approved. The companies priced this treatment, administered through infusion, at $56,000 a year.

The trial tested the drug only on early Alzheimer's patients, but the FDA put no restrictions on the label, leaving doctors to decide who to prescribe it to. Insurers haven't yet said they will cover the costs for all patients and whether brain imaging will be required initially or at intervals to show the level of plaque.

Alexander said doctors and patients will have to discuss the drug's potential benefits and the side effects, which could include brain

swelling, headaches and confusion.

"Patients and clinicians will have to navigate this together," he said.