New Alzheimer's Drug Brings Hope for the Future

When the Federal Drug Administration approved a new drug for Alzheimer's, relatives of those who suffer from the progressive neurological disease and those who worked on the clinical trials hailed it as a landmark decision.

"This is the first disease-modifying drug given to the public," says Barry Baumel, M.D., a neurologist with the University of Miami Health System. "This is the first treatment we have that actually slows the progression, not just treats the symptoms. It's the first to treat the underlying disease."

It's also the first therapy authorized in almost two decades for a disease that's proven to be particularly challenging to treat and manage. About 50 million people around the world suffer from the disease. In the U.S. alone, 6 million are living with Alzheimer's. In fact, one in three seniors dies with Alzheimer's or another dementia, which means that it kills more than breast cancer and prostate cancer combined. It is estimated that, in 2020, these diseases cost the country $305 billion.

Those numbers are growing. "Alzheimer's is a prevalent disease that is going to become more common as the population becomes older," Dr. Baumel adds. "That's
because age is the number one risk factor."

The Alzheimer's Association and other advocacy groups predict that by 2050 the number of Americans living with the disease will number about 14 million, and costs in caring for them will skyrocket to $1.1 trillion.

But it's not just the monetary cost that devastates families.

Watching a loved one deteriorate can be heartbreaking, so a rare approved treatment provides hope and a measure of comfort.

The new drug, a monoclonal antibody, is called aducanumab, but it will be marketed by Biogen as Aduhelm. It is given intravenously once a month and targets what many believe is the underlying cause of the neurodegenerative disease by reducing amyloid-beta plaques in the brain. (Amyloid forms abnormal clumps in Alzheimer-affected brains, which end up damaging cells.)

Dr. Baumel, who specializes in memory and cognitive disorders, was among the physician-scientists who participated in Phase III of the international clinical trials that involved 3,000 patients worldwide. Like his colleagues, he found that the patients who received aducanumab at the highest dose showed a reduction in amyloid in P.E.T. scans compared to those who received lower doses or a placebo. They also did better in cognitive measuring tests.

Not a cure, but a new tool in the treatment bag

Aduhelm is not a cure, says James Galvin, M.D., M.P.H., another neurologist with UHealth who also participated in the Phase III trials before coming to the University of Miami. It does, however, buy patients and their families much-appreciated extra time.
"Most drugs - I would say 99% - treat symptoms," Dr. Galvin explains. "With some exceptions, there aren't cures. But now [with Aduhelm], we have another tool in the treatment bag."

Finding treatment of any kind for Alzheimer's has been elusive. For one, the disease is difficult to diagnose. It also progresses over years, not months, and can vary tremendously from one patient to another. Until recently, almost 150 drug candidates were unsuccessful in clinical trials. In fact, between 1998 and 2017, only four drugs received F.D.A. approval - all of them treating symptoms, not slowing the progression. As a result, several drug manufacturers abandoned the work.

The same might have happened with Aduhelm if not for a more thorough review of the data. In March 2019, Biogen halted the late-stage trials of aducanumab when it appeared that the drug wasn't any better than a placebo. However, the company analyzed the data again, concluding that the treatment did work. Moreover, at higher doses, it slowed cognitive decline significantly.

Dr. Galvin believes the promise of Aduhelm might lure more researchers and pharmaceutical companies back to the field.

"This certainly provides hope," he adds.

While applauded in some sectors, the new Biogen treatment hasn't been without controversy. News of its approval focused on its hefty cost to consumers. With a $56,000 price tag, it is considered one of the most expensive drugs on the market. If private and public payers don't find a way to lessen the expense, it may make the drug inaccessible to many.

In addition, the path to F.D.A. was not a smooth one. Last fall, a panel of experts recommended that aducanumab not be approved because of doubt about its
effectiveness. The F.D.A., however, used a process known as "accelerated approval" to give the OK for the drug. The agency explained that the benefits of slowing Alzheimer's outweigh the risks. (This fast-track approval isn't unusual in the case of cancer drugs when consent is given to a drug "because it provides a meaningful therapeutic advantage over existing treatments.")

Drs. Baumel and Galvin agree with the F.D.A.'s decision, explaining that the drug gives precious extra time to patients. "Anyone who says this drug is coming out too soon isn't thinking of the disease but a statistic," he says. "A patient is not a statistic."

Dr. Baumel believes Aduhelm will become part of the standard of care for Alzheimer's, leading to more research on this particular class of monoclonal antibodies. "It's only going to improve the care we provide."

Moving forward, as part of the fast-track approval protocol, Biogen will have to perform what is known as Phase IV confirmatory trials. During these studies, researchers verify if the treatments are truly beneficial. If not, the F.D.A. can vote to take it off the market.

"We know that the first medicine approved in any class is rarely the best medicine," Galvin explains. "But there has to be a first before there is a best."

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Ana Veciana-Suarez, Guest Columnist
Ana is a regular contributor to the University of Miami Health System. She is a renowned journalist and author who has worked at The Miami Herald, The Miami News, and The Palm Beach Post. Visit her website at anavecianasuvelez.com or follow @AnaVeciana on Twitter.

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