FDA, Biogen, and an Alzheimer's drug approval: 8 key takeaways from congressional investigation



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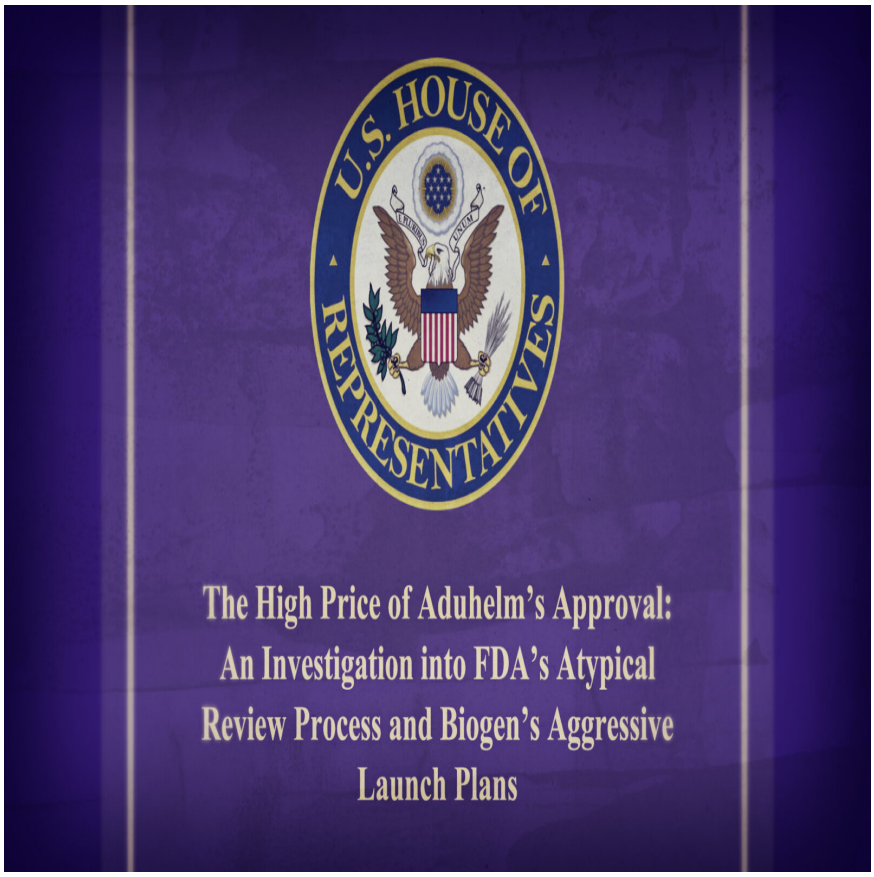

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Congressional investigators sorted through more than 500,000 pages of documents from the Food and Drug Administration and Biogen to chronicle how a controversial Alzheimer's disease drug was approved, and how it went from what was supposed to be the biggest drug launch in history to a financial catastrophe.

The resulting report[8] is filled with fresh details about how one of the most storied biotech companies plotted with the federal agency that is supposed to regulate it, and how Biogen decided to set a price for the drug, Aduhelm, that fueled a public outcry.

The investigation by two House committees was in part spurred by STAT's extensive reporting[9] and turned up many new revelations, which are detailed in these stories about Biogen's focus on maximizing profits[10] and FDA "irregularities."[11]

Here are eight key takeaways from the investigation:

FDA officials failed to document communications with Biogen

The FDA and Biogen formed a working group ahead of the company filing an application for Aduhelm's approval, and over a one-year period, investigators counted 115 meetings, calls, and substantive email discussions about Aduhelm. However, the committee revealed 66 calls and substantive email exchanges that weren't properly documented. FDA guidance says that the agency is supposed to document all "substantive" communications between the agency and drug sponsors.

Internal clashes caused last-minute chaos at the FDA

There were substantial internal disagreements over Aduhelm between two parts of the FDA: the Division of Biometrics, which does statistical reviews to ensure the safety and effectiveness of new drugs, and the Office of Neuroscience. The full extent of the dispute became apparent only about 10 days before an analysis for FDA advisers was due. FDA leadership scrambled, but ultimately weren't able to fully resolve the disagreements before the presentation, and the analysis critical of Aduhelm was only included as the very last appendix in a more than 300-page report.

Collaboration between the FDA and Biogen was 'atypical'

The FDA's and Biogen's joint preparation of a briefing document and a presentation for the FDA advisory committee meeting in November 2020 was unusual. It's an approach that the FDA had previously used only nine times, investigators wrote, and never before in the neuroscience office. Generally, the agency's and drug manufacturers' analyses and briefing documents are separate and independent. By contrast, Biogen worked so closely with the FDA on the briefing document that it was difficult to distinguish between who wrote what, congressional investigators found.

Biogen was banking on a blockbuster

Facing challenges to its business, Biogen's plan was to "make history" with Aduhelm and establish the drug as "one of the top pharmaceutical launches of all time." Internal projections forecasted sales to reach $1 billion within one year after approval, and peak at $23 billion annually. "Our top and bottom line will quickly rebound with the launch of" Aduhelm, a 2020 company presentation read, adding that a successful launch would represent a "change of magnitude for Biogen and humanity."

The company was 'pushing the limit' on price

More than a year before Aduhem's June 2021 approval, outside consultants, insurers, and physicians advised Biogen to consider a moderate price for Aduhelm in the range of $15,000 to $30,000 in order to maximize the drug's value and enable access to the most patients. Anything above $40,000, according to one internal document, would be "pushing the limit" of what the system would tolerate. But Biogen chose to price Aduhelm at $56,000 a year to prioritize "revenue maximization."

Biogen knew Aduhelm would be a budget-breaker

The financial burden for Aduhelm would largely fall on Medicare, Biogen acknowledged in an internal projection. Roughly 85% of potential patients would be Medicare beneficiaries. Even if just 250,000 patients were to receive the drug, it would cost Medicare more than $12 billion a year, which would be 26% of the program's annual budget for medicines administered in a physician's office and nearly five times as much as the next costliest drug. The company knew many patients would struggle to afford Aduhelm, the documents show. Biogen's analysis estimated some Medicare patients would face out-of-pocket costs of up to 20% of their income.

The FDA proposed allowing Aduhelm for all Alzheimer's patients

The congressional investigation revealed for the first time that the FDA had initially recommended an unusually broad label for Aduhelm. The original label made all patients with Alzheimer's eligible to receive the drug, even though it had been tested only on patients with early-stage disease. Despite internal misgivings, Biogen didn't push back on the broad label, documents showed. After a month of intense backlash, Biogen made a request to narrow the label to the population studied in clinical trials.

Biogen aimed to target communities of color with marketing, not clinical data

Biogen was focused on marketing Aduhelm to people of color, planning to spend upward of $3 million to advertise on Telemundo and BET and setting out another $1 million for multilingual patient outreach, according to internal presentations. But "Biogen's proposed health equity narrative and marketing plans were not supported by Aduhelm's clinical trials," the government investigation noted. In the two pivotal studies used to win FDA approval, only 3% of participants were Hispanic and 0.6% were Black.

The Aduhelm Files also include what the House report reveals about Biogen's ambition to make Aduhelm a "blockbuster"[10] *and the FDA's role*[11] *in approving the controversial drug. Read the full congressional investigation report here*[8].

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