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## In new remarks, Cassava Sciences' CEO shifts defense of embattled treatment for Alzheimer's



By [Adam Feuerstein](#) Sept. 13, 2021



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Cassava Sciences CEO Remi Barbier on Monday shifted his defense of the company's embattled treatment for Alzheimer's disease: The drug is safe, he said, therefore the Food and Drug Administration shouldn't interfere with the company's plan to start late-stage clinical trials.

Barbier, speaking via a prerecorded video during an investor conference sponsored by the investment bank H.C. Wainwright, also backtracked from claims made by Cassava that an ongoing mid-stage study showed its drug had improved the cognition of patients with Alzheimer's.

A [citizen petition filed with the FDA](#) last month accused Cassava of altering Western blot images and laboratory biomarker data that support its drug, called simufilam. The petition, filed by a law firm representing unnamed whistleblowers, asked the agency to halt all simufilam clinical trials and investigate the alleged data manipulation.

Cassava has called the allegations made by the whistleblowers "false," but has so far declined to back up its defense of the drug with concrete evidence. It also admitted some errors in previous simufilam data presentations.

Barbier has blamed short sellers for trying to derail the company's efforts at developing an effective drug for Alzheimer's. His comments Monday were notably less combative, even conciliatory. Barbier was speaking directly to investors, but, indirectly, he seemed to be signaling the FDA officials who are now considering the merits of the citizen petition — and whether or not to halt simufilam clinical trials.

"In my experience, a credible citizen petition usually focuses around clinical data," said Barbier on Monday. "If you take a step back, what is the FDA's concern? The FDA's currency is clinical data, particularly safety. Safety is all

important. The FDA exists in order to protect people, patients. Our drug to date has had a safe profile in an elderly, somewhat frail population. I don't see the safety argument as being credible.”

It's true that the citizen petition doesn't make specific claims against the safety of simufilam, and no data presented to date suggest the drug is causing potentially dangerous side effects. But critics say the FDA could — and should — halt clinical trials if Cassava and its collaborators are found to have falsified data submitted to the agency to justify past and future studies in patients.

Barbier's comments were a “total non-sequitur,” [tweeted](#) Jacob Sherkow, a professor specializing in patent law and bioethics at the Illinois College of Law. “I don't think the complaint is a fear that the drug isn't ultimately \*efficacious.\* It's that the IND was seemingly obtained from fraudulent data.” Sherkow is not involved in the citizen petition.

Last [February](#) and then again in [July](#), Cassava issued press releases in which it claimed simufilam was improving the cognition of patients with Alzheimer's — a benefit that no other drug has ever shown. The findings were based on preliminary analyses of a small clinical trial conducted without a placebo control arm, suggesting patients who knew they were being prescribed an experimental treatment could have been biased to report more positive scores on subjective cognitive tests. Independent experts in Alzheimer's disease have [criticized Cassava for exaggerating](#) simufilam's cognitive benefit, based on these data.

On Monday, Barbier walked back the company's previous statements.

“We don't have efficacy, nor do we claim we have efficacy because human efficacy in a drug candidate can only be proven by the FDA standard — a Phase 3 clinical program,” he said. “We think this drug candidate is worthy of a Phase 3 clinical program. Others others may disagree. That's OK that's an opinion, but we think what we're seeing is very directional and very consistent.”

The FDA can take up to six months to rule on the merits of a citizen petition, although the agency can issue a decision earlier or later, depending on other factors. The CUNY School of Medicine in New York is also investigating the allegations of simufilam research misconduct related to the published papers of Hoau-Yan Wang, a tenured medical professor and scientific adviser to Cassava.

Cassava's stock price has fallen 68% since the citizen petition was filed. Shares were down another 4% Monday following Barbier's remarks.

## About the Author



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