

United States Senate

WASHINGTON, DC 20510

August 9, 2019

The Honorable Norman E. “Ned” Sharpless, M.D.
Acting Commissioner
United States Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, Maryland 20993

Dear Acting Commissioner Sharpless:

On August 6, 2019, public reports emerged that AveXis, a subdivision of pharmaceutical giant Novartis, falsified data to the Food and Drug Administration (FDA) to obtain approval of its gene therapy, Zolgensma. This new biological product is the most expensive medication in American history, with a price tag of \$2.1 million. This scandal smacks of the pharmaceutical industry’s privilege and greed, and Americans are sick of it.

We are committed to the development of innovative treatments for patients, and have worked to increase biomedical research funding at the National Institutes of Health (NIH) by 30 percent over the past four years. In fact, federally funded NIH research contributed to the development of Zolgensma. On May 24, 2019, the FDA approved Zolgensma for children with a severe form of spinal muscular atrophy, which is an important medical breakthrough for a vulnerable patient population. However, AveXis failed to disclose to the FDA the inaccuracy of its product testing data until June 28, 2019—despite apparently having prior knowledge of the issue in March. It is an outrage that after knowingly misleading the FDA in a rush to make a profit, this pharmaceutical company refused, in its August 6, 2019, press release, to acknowledge any culpability or remorse. Instead, the company doubled down on its greed-driven behavior.

What makes this unscrupulous action even more appalling is the fact that AveXis was the beneficiary of numerous federal taxpayer-funded benefits and incentives, including obtaining Fast Track, Breakthrough Therapy, and Priority Review designations—ensuring that Zolgensma would be sped through the regulatory approval process. Further, AveXis was awarded a rare pediatric disease priority review voucher—which is worth tens of millions of dollars. AveXis also benefited from taxpayer-funded research at the NIH for comparative studies on patients with spinal muscular atrophy. All the while, AveXis breaks records, and budgets, with its staggering \$2.1 million price.

It is unconscionable that a drug company would provide manipulated data to federal regulators in order to rush its product to market, reap federal perks, and charge the highest amount in American history for its medication. Such greed cannot be condoned by the FDA. We urge you to use your full authorities to hold AveXis accountable for its malfeasance, including through all appropriate criminal, civil, and regulatory actions against the company. Anything short of a forceful response would signal a green light to future pharmaceutical misbehavior.

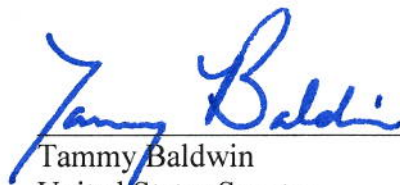
We also request a formal written explanation by September 1, 2019, for why FDA decided to withdraw a proposed regulation in October 2018 (RIN 0910-AC59), which would have required sponsors of certain clinical trials to promptly report suspected data falsification to FDA. In light of AveXis' alarming actions, we also seek to know whether FDA plans to re-issue such regulation. FDA's justification for the proposed rule was because "it is important for the agency to have confidence in any data from studies." American patients also want to know whether they can trust the integrity of FDA's approval process.

Thank you for your attention to this situation, and your efforts to ensure patient safety.

Sincerely,



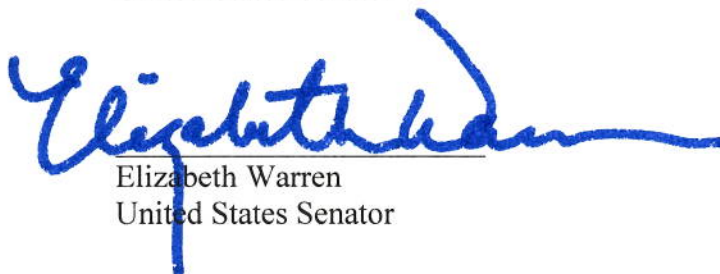
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