

Data Manipulation Scandal Rocks the World's Most Expensive Drug



By Christopher Gerry — August 19, 2019

Zolgensma, the gene therapy, developed and marketed by the pharmaceutical company Novartis, won both FDA approval and the title of "world's most expensive drug" earlier this year. However, it's recently come to light that Novartis knowingly provided the FDA manipulated data during the approval process. Dr. Chris Gerry explains what went wrong, why this is such a big deal, and whether Zolgensma should stay on the market.



Image: Aid Leap [1]

When newly minted physicians take the [Hippocratic Oath](#) [2], they are committing to a set of values that will guide their professional lives. As you might be aware, those principles can be summarized neatly by the phrase “first, do no harm.” **(1)**

No such code of ethics applies to Ph.D. scientists. But if one did exist, I imagine it would sound something like: “first, do not mess with the data.” And for drug developers, an obvious corollary would read: “second, do not mess with the FDA.”

The Swiss pharmaceutical giant Novartis appears to have broken both of those golden rules. Last week, it was reported that Novartis had [submitted manipulated data](#) [3] to the FDA during the approval process for the gene therapy Zolgensma, [the world's most expensive drug](#) [4] at \$2.125

million. Even worse, [Novartis knew](#) ^[5] about the data manipulation before Zolgensma won approval but didn't mention it to the FDA until [over a month later](#) ^[6]. And while Vas Narasimhan, Novartis' CEO, is claiming that the company can be "[proud](#) ^[7]" of the way it handled the situation, both the [FDA](#) ^[8] and [high-profile politicians](#) ^[9] have taken several shots at Novartis, putting their full bodyweight into every punch.

Though still very serious—more on that in a minute—the fraudulent data thankfully does not compromise the safety or efficacy of Zolgensma in patients. The experiments in question were performed in mice (*i.e.* not humans) and related to the production process of the drug (*i.e.* not its effects in the clinic). As a result, the FDA "[remains confident that Zolgensma should remain on the market](#) ^[8]." This response makes sense—Zolgensma's jaw-dropping results in children with the otherwise fatal disease spinal muscular atrophy are very real. Still, it's likely that the agency would have [delayed](#) ^[10] the approval had it learned about the problematic data earlier.

But this is not a case in which the ends justify the means—"no harm, no foul" doesn't apply. Our collective understanding of biology, chemistry, medicine, and their various junctures is largely built upon trust. When a scientist reads a paper in a scientific journal, she may quibble with the authors' conclusions given the data that are presented, but she won't get very far if she believes that those data are simply made up. As chemist and blogger Dr. Derek Lowe has put it, "[the scientific enterprise assumes that you are not lying about everything](#) ^[11]."

For some, it may seem heretical that faith (in fellow scientists) plays such an important role in the scientific process. Though far from ideal, it's the only logical outcome when [millions of articles](#) ^[12] are published every year in tens of thousands of different journals. There simply isn't enough time in the day or money in the bank to attempt to reproduce every experiment systematically. And even when inconsistencies do bubble up, it can sometimes take years before a correction or retraction attempts to [revise the scientific record](#) ^[13].

The mechanics involved in pruning the scientific literature don't extend to the world of drug discovery because the stakes are too high (as measured in both dollars and lives). Errors must be caught and corrected immediately, otherwise patients could pay for useless or harmful medications. Therefore, the academic tools of journal editors and peer review are supplanted by the FDA, clinical trials, and [New Drug Applications](#) ^[14] that require data collected from every point in the candidate drug's lifetime.

Though ultimately inconsequential from a clinical standpoint, the scandal surrounding Zolgensma has exposed important vulnerabilities in the system that [need to be fixed](#) ^[15]. We simply cannot have drug companies submitting questionable data of any kind when applying for a new drug approval. Nothing can be left to chance; nothing can be taken on faith.

(1) Fun fact: this phrase doesn't actually appear in the Hippocratic Oath, either in the (translated) original or modernized versions. Go figure...

Source URL: <https://www.acsh.org/news/2019/08/19/data-manipulation-scandal-rocks-world%E2%80%99s-most-expensive-drug-14230>

Links

[1] <https://aidleap.org/2015/05/17/why-dont-international-medical-ngos-follow-the-hippocratic-oath/>

[2] https://www.nlm.nih.gov/hmd/greek/greek_oath.html

[3] <https://www.msn.com/en-us/health/health-news/fda-says-some-data-used-to-approve-worlds-most-expensive-drug-was-faulty/ar-AAFqWvw>

[4] <https://www.acsh.org/news/2019/05/28/science-zolgensma-world%E2%80%99s-most-expensive-medicine-part-1-14053>

[5] <https://www.statnews.com/2019/08/06/novartis-was-aware-of-manipulation-of-data-supporting-gene-therapy-approval-fda-says/>

[6] <https://www.fiercepharma.com/pharma/sanders-warren-join-team-senators-blasting-novartis-zolgensma-data-rig>

[7] <https://www.statnews.com/2019/08/07/novartis-ceo-we-tried-to-do-the-right-things-in-fda-data-scandal/>

[8] [https://www.fda.gov/news-events/press-announcements/statement-data-accuracy-issues-recently-approved-gene-](https://www.fda.gov/news-events/press-announcements/statement-data-accuracy-issues-recently-approved-gene-therapy?utm_campaign=080619_Statement_Statement%20by%20FDA%20on%20data%20accuracy%20issues%20wi)

[therapy?utm_campaign=080619_Statement_Statement%20by%20FDA%20on%20data%20accuracy%20issues%20wi](https://www.fda.gov/news-events/press-announcements/statement-data-accuracy-issues-recently-approved-gene-therapy?utm_campaign=080619_Statement_Statement%20by%20FDA%20on%20data%20accuracy%20issues%20wi)

[9] <https://www.businessinsider.com/novartiss-zolgensma-data-manipulation-gets-scrutiny-from-us-senators-2019-8>

[10] <https://www.nytimes.com/2019/08/06/health/novartis-fda-gene-therapy.html>

[11] <https://blogs.sciencemag.org/pipeline/archives/2019/07/30/startups-and-their-publications>

[12] <https://www.smithsonianmag.com/smart-news/half-academic-studies-are-never-read-more-three-people-180950222/>

[13] <https://www.theatlantic.com/health/archive/2012/12/the-myth-of-self-correcting-science/266228/>

[14] <https://www.fda.gov/drugs/types-applications/new-drug-application-nda>

[15] <https://news.bloomberglaw.com/pharma-and-life-sciences/fda-wont-require-study-sponsors-to-report-suspected-misconduct-1>