

Zolgensma: More Musings on the Price of the World's Most Expensive Drug (Part 3/3)



By Christopher Gerry — June 13, 2019



According to *The Picture of Dorian Gray* by Oscar Wilde, “there is only one thing in the world worse than being talked about, and that is not being talked about.”

I think a certain Swiss pharmaceutical company would beg to differ.

Novartis recently won FDA approval for [Zolgensma](#) ^[1], a new medicine that can potentially cure infants suffering from the otherwise fatal disease spinal muscular atrophy (SMA). But its record-high price of \$2.125 million has sparked a broader conversation about drug pricing characterized by [bemused practicality](#) ^[2], [scathing indignation](#) ^[3], and everything in between.

I wrote about the [science](#) ^[4] behind Zolgensma a few weeks ago, and since then I've been examining common justifications for its unprecedented price tag. I've already concluded that the need to recoup R&D costs is [not a viable excuse](#) ^[5]. This week's article will explore two other oft-heard answers to the question of the moment: why is the price of Zolgensma so high?

Answer #2: Zolgensma is a bargain compared to the standard of care for SMA.

Though the numbers are big, this looks like a straightforward math problem. The only other treatment for SMA is a drug called Spinraza (made by Biogen). But unlike Zolgensma, which is administered just once, Spinraza must be taken for life. The first year of Spinraza costs [\\$750,000](#) ^[6], and all subsequent years cost \$375,000, so five years of Spinraza (\$2.25 million) costs more than one dose of Zolgensma (\$2.125 million). Therefore, if Zolgensma proves to be as effective as we all hope it to be—one dose for decades of therapeutic benefit—then it's clearly the least expensive way to treat SMA.

But “least expensive” is a relative term. \$2.125 million is an astounding sum that only looks

reasonable when compared to the prices of other healthcare products and services. The United States spends an [exorbitant amount](#) [7] on healthcare, and the high price for prescription drugs—especially [compared to other countries](#) [8]—is a one of the primary contributors. Therefore, how meaningful is it that Zolgensma is the most cost-effective treatment for SMA? How well can the one-eyed man see, even if he is king of the blind?

Answer #3: The ability to set a high price for new drugs incentivizes biomedical innovation.

It takes a lot of time and money to bring a new drug to the market, and the most innovative therapies often rely upon years—if not decades—of basic research from both industry and academia. How, then, do you encourage drug hunters to work on medicines that will treat just a handful of patients with an exceedingly rare but debilitating disease?

The current system in the United States offers several financial incentives. The Orphan Drug Act of 1983, for example, established [a tax credit](#) [9] for companies that develop new drugs for diseases that affect less than 200,000 patients. But the federal government’s biggest impact stems from its granting of [patents](#) [10] for new medicines, allowing holders to behave as *de facto* monopolists for ~20 years **(1)**. As a result,

pharmaceutical companies can price their new drug at a level that will maximize revenue without worrying about competition (as I discussed [last week](#) [5]).

These incentives appear to be working, but perhaps too well. [More than 200 drugs](#) [11] for rare diseases have been approved since 1983, signaling an industry-wide [shift in focus](#) [12]. The only way that these drugs can make money, however, is with high prices—there just aren’t enough patients. In the case of Zolgensma, insurers are [expected to cover](#) [13] most (if not all) of its cost, but that just establishes a new baseline for what’s considered tolerable. At what price does innovation become too costly?

So, what’s the truth? Is the price of Zolgensma too high, or is it justified? If there’s one thing this exercise has taught me so far—and if I haven’t used up my allowance of Oscar Wilde quotes already—it’s that “the truth is rarely pure and never simple.”

NOTES:

(1) You won’t be surprised to learn that patent law in the pharmaceutical industry is [maddeningly complex](#) [14]. The 20-year clock starts on the date that the patent filed, which (almost) always happens before the drug is first tested in clinical trials. Human testing and FDA review can take upwards of 10 years, so companies often have much less than their original 20-year allotment when the drug first hits the market. But various combinations of high-paid patent attorneys—many of whom have PhDs in science—and government provisions can extend the life of a patent. And when lawsuits are brought, the court system isn’t exactly known for its speed. Like I said, it’s complicated.

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Links

[1] <https://www.statnews.com/2019/05/24/hold-novartis-zolgensma-approval/>

[2] <https://www.fool.com/investing/2019/05/31/industry-focus-healthcare-05-29-2019.aspx>

[3] <https://www.bloomberg.com/opinion/articles/2019-05-24/check-the-exorbitant-price-of-zolgensma-and-other-orphan-drugs>

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

[5] <https://www.acsh.org/news/2019/06/05/dollars-and-sense-how-think-about-price-zolgensma-part-2-14074>

[6] <https://www.cbsnews.com/news/the-cost-of-biogens-new-drug-spinraza-750000-per-patient/>

[7] <http://fortune.com/2019/02/21/us-health-care-costs-2/>

[8] <https://www.scientificamerican.com/article/how-the-u-s-pays-3-times-more-for-drugs/>

[9] <http://sitn.hms.harvard.edu/flash/2018/risky-business-far-reaching-consequences-slashing-orphan-drug-tax-credit/>

[10] <https://www.news-medical.net/health/Drug-Patents-and-Generics.aspx>

[11] <https://www.bio.org/articles/impact-orphan-drug-tax-credit-treatments-rare-diseases>

[12] <https://www.the-scientist.com/features/how-orphan-drugs-became-a-highly-profitable-industry-64278>

[13] <https://www.npr.org/sections/health-shots/2019/05/24/725404168/at-2-125-million-new-gene-therapy-is-the-most-expensive-drug-ever>

[14] <https://www.fda.gov/drugs/development-approval-process-drugs/frequently-asked-questions-patents-and-exclusivity>