Viewpoint: Spinal muscular atrophy treatment Zolgensma a gene therapy trailblazer, despite controversy

In August, the US Food and Drug Administration (FDA) gave drug manufacturer AveXis, Inc, a subsidiary of Novartis AG, a major slap on the wrist for violations related to the approval of Zolgensma, a new treatment for spinal muscular atrophy (SMA). The agency said the company had failed to promptly report to the proper regulatory authorities issues of data manipulation in some product testing. Ominous newspaper headlines followed.

It didn't help that the drug is extremely expensive—a record-setting $2.1 million for a single dose.

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But some of the regulatory concerns, at least, seem to be overblown. According to the FDA's inspection report, the initial data discrepancies were limited to a handful of mouse experiments, and importantly, the human clinical results look sound.

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In the eyes of the public and politicians, the reputation of Zolgensma—the first gene replacement therapy to hit the market for a neurological disease—may be tarnished, at least in the near term. But the flurry of media coverage may also have obscured what the drug development community believes to be the more enduring story of Zolgensma: its impact not only on patients but on the entire field of gene therapy.

Read full, original post: News Feature: Gene therapy successes point to better therapies