NEWS

# U.S. government plans to probe orphan drug program

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Acting on a request from three influential U.S. senators, the government's accountability arm confirmed that it will investigate potential abuses of the Orphan Drug Act.

The Government Accountability Office (GAO) still must determine the full scope of what it will look into and the methodology to be used. Determining the scope will take some months, says Chuck Young, GAO's managing director for public affairs.

Earlier this month, senators Orrin Hatch (R-Utah), Chuck Grassley (R-Iowa) and Tom Cotton (R-Arkansas) sent a **letter** to the GAO and raised the possibility that regulatory or legislative changes might be needed "to preserve the intent of this vital law" that gives drugmakers lucrative incentives to develop drugs for rare diseases.

Grassley's office says they expect the GAO to begin its work in about nine months. The delay is typical as the agency has a queue of requests it is pursuing.

The senators have asked the GAO to "investigate whether the ODA is still incentivizing product development for diseases with fewer than 200,000 affected individuals, as intended."

Congress overwhelmingly passed the 1983 Orphan Drug Act to motivate pharmaceutical companies to develop drugs for people whose rare diseases had been ignored. Drugs approved as orphans are granted tax incentives and seven years of exclusive rights to market drugs that are needed by fewer than 200,000 people in the United States.

In recent months, reports of five- and six-figure annual price tags for orphan drugs have amplified long-simmering concerns about abuse of the law. The senators' call for a GAO investigation

reflects that sentiment.

“While few will argue against the importance of the development of these drugs, several recent press reports suggest that some pharmaceutical manufacturers might be taking advantage of the multiple designation allowance in the orphan drug approval process,” the letter states.

## Maximizing profits:

In January, *Kaiser Health News* published an **investigation** that found the orphan drug program is being manipulated by drugmakers to maximize profits and to protect niche markets for medicines being taken by millions.

That investigation, which also was **published and aired** by NPR, found that many drugs that now have orphan status aren’t entirely new. More than 70 were drugs first approved by the U.S. Food and Drug Administration (FDA) for mass-market use. Those include cholesterol blockbuster Crestor, Abilify for psychiatric disorders and the rheumatoid arthritis drug Humira, the world’s best-selling drug.

Others are drugs that have received multiple exclusivity periods for two or more rare conditions.

The senators asked the GAO for a list of drugs approved or denied orphan status by the FDA. It also asked if resources at the FDA, which oversees the law, have “kept up with the number of requests” from drugmakers and whether there is consistency in the department’s reviews.

And they said it would be important to include patient experiences in the GAO review. The GAO does not provide updates on ongoing work, but rather reports its findings once it completes an assignment.

The rare-disease drugs have become increasingly popular with pharmaceutical and biotech companies. They are expected to constitute 21.4 percent of worldwide prescription sales by 2022, not including generics, according to consulting firm EvaluatePharma’s 2017 orphan drug report.

That’s in part because of the exorbitant prices that can be charged. Of the top 100 drugs in the U.S., the average cost per patient per year for an orphan drug was $140,443 in 2016, compared with $27,756 for a non-orphan, EvaluatePharma said.

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