**(PBS News Hour) How one drug company fast-tracked the FDA’s review process**

SEPTEMBER 30, 2016

[Article source](http://www.pbs.org/newshour/rundown/one-drug-company-fast-tracked-fdas-review-process/) [PBS Newshour](http://www.pbs.org/newshour/rundown/one-drug-company-fast-tracked-fdas-review-process/)

*By Sarah Jane Tribble, Kaiser Health News*

In 2007, Congress created a voucher program that shaves months off of the Food and Drug Administration’s review process for drug development that targets rare pediatric diseases.

Dugmaker Sarepta Therapeutics won a big victory when its $300,000 muscular dystrophy drug was recently approved, but the company had other reasons to celebrate, too.

They were also awarded the drug world’s equivalent of a Willy Wonka golden ticket.

The ticket, known as a rare pediatric disease priority review voucher, is part of a program created by Congress in 2007 to encourage the development of drugs for tropical diseases and later expanded to rare pediatric disorders. Any company awarded a voucher can use it for a fast-track government review of one of its future drugs — or it can sell the voucher to another company.

“The only people who would buy a priority review voucher would be someone who had something that wouldn’t merit its own priority review but they want the priority review,” says Dr. Tim Coté, a former FDA official who now runs a consulting firm for rare disease drugs.

In other words, the companies willing to pay for a voucher are most likely trying to get a medicine that treats a common disease on the market quickly for competitive reasons, he said.

“It might be a blockbuster — say a new statin,” Coté said, referring to a drug that lowers cholesterol. “A priority review would make all the difference in the world.”

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And the sale of one voucher can earn hundreds of millions of dollars for a small company like Sarepta. Last year, a voucher sold for a record $350 million when AbbVie Inc. bought one from United Therapeutics. The company has not announced plans for the voucher.

It’s those price tags — as well as admonishments from the Food and Drug Administration — that have some industry watchers debating whether the program should continue. The House voted Tuesday to extend the program until the end of the year, after the Senate had already done so. It was set to expire this month.

Members of Congress, the pharmaceutical industry, and rare disease advocates have passionately supported the voucher program as a way to incentivize rare disease drug development. The goal, they say, is to steer more money toward rare disease drug developers by allowing them to sell the voucher to the highest bidder. But others, such as FDA officials and [academics](http://healthaffairs.org/blog/2016/06/15/are-priority-review-vouchers-the-answer-to-incentivize-drug-development-not-so-fast/), have questioned whether the program is leading to any new drugs for rare diseases.

Nancy Goodman, executive director of Kids v Cancer, is a champion of the bill. She created her foundation after her son died seven years ago of medulloblastoma, a rare pediatric brain cancer.

There were no drugs available for her 10-year-old son, she said.

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The legislation extending the program passed the House and Senate by unanimous consent and awaits action by President Barack Obama, who is expected to sign it. Advocates are fighting to keep the program authorized until Dec. 21, 2018 as part of the [21st Century Cures Act](https://energycommerce.house.gov/cures), which has passed the House and has stalled in the Senate, where backers hope it will be taken up after the elections.

But officials at the FDA, which must implement the vouchers, are at odds with industry and advocates.

Dr. John Jenkins, director of the office of new drugs in the Center for Drug Evaluation and Research at the FDA, has said he supports the overall goal of providing incentives to promote drug development but that the voucher program is wrong.

In December, Jenkins told a reporter at the industry publication Pharmaceutical Executive that vouchers raise safety concerns because the program requires the FDA to give any drug accelerated review — even when reviewers have to address complex issues.

“We’re not making pizza here,” he said.

Jenkins declined interview requests for this story. FDA spokeswoman Sandy Walsh said, the “FDA has not seen evidence that the program is effective.”

Walsh also said the agency is concerned that the voucher program “adversely affects the agency’s ability to set its public health priorities” and “the additional workload from the program strains the agency’s resources.”

The voucher program does require companies to pay a user fee of nearly $3 million to help the agency pay for the program. But the additional money may not be sufficient.

In March, a Government Accountability Office report said FDA officials complain there is too little time to use the money to hire and train additional employees to do accelerated reviews and that the one-time fee does not enable hiring of long-term staff.

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Of the seven pediatric review vouchers that have been awarded, four have been sold to other drug companies.

Drugmaker Sanofi has redeemed two of the vouchers. For the first, they paid $67.5 million and used it to expedite review of their cholesterol drug Praluent. Later, Sanofi spent $245 million for a voucher and said in February it would be used to speed up the FDA’s decision on a new type 2 diabetes treatment.

If a voucher is not used, it can be sold an unlimited number of times before being used.

Sarepta plans to sell its voucher, Chief Financial Officer Sandy Mahatme told Wall Street analysts in announcing approval of its drug to treat Duchenne muscular dystrophy. He said the money would help finance other drugs in its pipeline, pay for the scaling up of its manufacturing and support the company’s entry into European markets.

Mahatme said they have already reached out to “a bunch of potential buyers.”