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RARE DISEASES

Too Early to Gauge Effectiveness of FDA's Pediatric Voucher Program

GAO Highlights

Highlights of GAO-16-319, a report to congressional committees

Why GAO Did This Study

Almost 7,000 rare diseases, most of which are serious or life-threatening, affect more than 25 million Americans. About half of all rare diseases affect children, and few of these diseases have viable treatments. To encourage the development of drugs to treat or prevent rare pediatric diseases, the Food and Drug Administration Safety and Innovation Act (FDASIA) of 2012 authorized FDA to award a priority review voucher to a drug sponsor upon approval of that sponsor's drug to treat a rare pediatric disease. A drug sponsor can later redeem the voucher when submitting another new drug application to treat any disease or condition in adults or children, or sell or transfer the voucher to another sponsor. A voucher entitles a sponsor to a 6-month priority review by FDA rather than the 10-month standard review.

FDASIA included a provision for GAO to study the pediatric voucher program. GAO examined what is known about the effectiveness of the program in encouraging the development of drugs to prevent or treat certain rare pediatric diseases. GAO reviewed relevant laws and documentation related to the program and its management, and identified drug sponsors who were awarded vouchers, the diseases their drugs were approved to treat, and whether the vouchers were redeemed, sold, or transferred. GAO also interviewed FDA officials, drug sponsors, patient advocacy groups, and organizations representing physicians and children's hospitals, among others.

View GAO-16-319. For more information, contact Marcia Crosse at (202) 512-7114 or crossem@gao.gov.

What GAO Found

It is too early to gauge whether the Food and Drug Administration's (FDA) pediatric voucher program has stimulated the development of drugs to treat or prevent rare pediatric diseases. Given that the typical drug development process often exceeds a decade, insufficient time has elapsed to determine whether the 3 year-old program has been effective. Any drug sponsors motivated by the program to attempt to develop a drug for a rare pediatric disease may be many years from submitting new drug applications—which contain scientific and clinical data about safety and effectiveness—to FDA for review.

As of December 31, 2015, there have been 11 requests for a pediatric voucher. Of these, six have been awarded, two denied, and three remain under review. The six drugs for which vouchers were awarded were in development prior to the program's implementation and these drugs helped fulfill unmet medical needs. One drug is indicated to treat a rare pediatric cancer, and the other five drugs treat rare metabolic diseases affecting children. No other drugs had been previously approved by FDA for these conditions. Four of the six awarded pediatric vouchers have been sold to other drug sponsors for prices ranging from $67.5 million to $350 million. One of the six vouchers awarded has been redeemed and was used to obtain a priority review of a new drug application for a drug to treat adults with high cholesterol. FDA approved this new drug application in July 2015.

FDA officials stated that, while they strongly support the goal of incentivizing drug development for rare pediatric diseases, they have seen no evidence that the program is effective. The program's authorization, as amended, is set to terminate October 1, 2016, and FDA officials said they do not support the program's continuation. They expressed concern that the program adversely affects the agency's ability to set its public health priorities by requiring FDA to provide priority reviews of new drug applications that would not otherwise qualify if they do not treat a serious condition or provide a significant improvement in safety or effectiveness. Additionally, FDA officials said that the additional workload from the program strains the agency's resources. However, other stakeholders provided generally positive feedback on the program. For example, drug sponsors that sold these vouchers said they plan to reinvest portions of the proceeds they received into additional research on rare pediatric diseases, although there is no requirement to do so. Patient advocacy groups told GAO that the program could lead to the development of needed drugs.

We provided a draft of this report for comment to the Department of Health and Human Services (HHS). HHS provided technical comments, which we incorporated as appropriate.