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United States Senate

COMMITTEE ON FINANCE

WASHINGTON, DC 20510-6200

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July 30, 2013

Mary K. Wakefield, BSN, MS, PhD
Administrator
Health Resources and Services Administration
Parklawn Building
5600 Fisher's Lane, Room 14-05
Rockville, MD 20857

Dear Administrator Wakefield:

The *Exclusion of Orphan Drugs for Certain Covered Entities under 340B Program* final rule implementing the requirement under the Patient Protection and Affordable Care Act (PPACA) to exclude orphan drugs under section 526 of the Federal Food, Drug and Cosmetic Act (FFDCA) for certain covered entities is not only incorrectly interpreting the law, but diminishes the incentives provided under the Orphan Drug Act.

First, the 340B exclusion provided to orphan drugs under section 2302 of PPACA clearly states that "...the term covered outpatient drug shall not include a drug designated under section 526..." of the FFDCA. The statute does not say the exclusion only applies when the product is used for the orphan disease or condition, but if the product is designated. The final rule limits this exclusion to "orphan drugs that are transferred, prescribed, sold or otherwise used for the rare condition or disease," completely ignoring the specific language of the law.

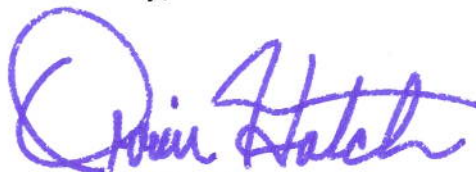
Second, the final rule establishes implements the law in a manner that the agency itself has proven it cannot enforce. HRSA has a long and storied history of lacking the capacity to conduct appropriate oversight of programs under the agency's jurisdiction, including Community Health Centers, the Ryan White program and more importantly the 340B program itself. If the agency has been ineffective in administering simple program integrity activities such as recertification of eligible covered entities, identifying drug diversion and ensuring 340B pricing is provided to covered entities, how will it track when a drug is used for an orphan disease or condition to ensure the manufacturers and covered entities are complying with the final rule? We know that HRSA cannot track those purchases with any confidence, which subjects the program to the honor system – a standard that we have seen before in this Administration that lacks credibility or any assurance of program integrity.

Third, the final rule states that marketing exclusivity for orphan drugs only applies when the orphan drug is targeted or used to treat the rare disease, suggesting that exclusivity only applies when a health professional or patient administers or uses the drug for the specific treatment of an orphan disease or condition. This is incorrect. Furthermore, the final rule suggests that incentives, including exclusivity, “do not apply to any indication for a disease or condition that has not itself received orphan drug designation,” and that the product would not be considered an “orphan drug’ for such additional uses.” To be clear, under the Orphan Drug Act, the Food and Drug Administration (FDA) is prohibited from approving another company’s application for the same product for the same orphan indication, but it does not regulate exclusivity through the “use” of the drug to treat a rare disease. To suggest that the exclusivity provided under the Orphan Drug Act is limited to justify the narrow interpretation of the exclusion under the 340B program is factually inaccurate and misleading.

Finally, the final rule establishes a new policy that erodes the incentive provided to manufacturers under the Orphan Drug Act to develop therapies that are intended to treat, diagnose, or prevent a rare disease that affects fewer than 200,000 people; or that affects more than 200,000 persons but are not expected to recover the costs of developing and marketing the product. The narrow application of the final rule will diminish the value of the incentives provided under the Orphan Drug Act and will result in fewer investments in products intended to treat rare diseases.

We understand the critical importance of balancing the needs of providers and manufacturers to ensure patients are able to access affordable and innovative products that will meet the needs of individuals with rare diseases and conditions. It is unfortunate that the consequences of the final rule will be borne by the patients the 340B Program and Orphan Drug Act were intended to protect.

Sincerely,



Orrin G. Hatch
Ranking Member
Senate Committee on Finance