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'Orphan drug' rule raises off-label worries for drugmakers

By Terry Baynes

(Reuters) - The U.S. Department of Health and Human Services last week clarified when hospitals are allowed to buy so-called orphan drugs at sharply discounted prices, but it left open questions for drug manufacturers about the risk of being prosecuted for off-label promotion of them.

The drugs, which are for rare diseases and named after the small "orphan" populations of fewer than 200,000 people they're designed to treat, are among the most expensive on the market.

In a long-awaited final rule issued on July 23, the department's Health Resources and Services Administration (HRSA) increased the number of hospitals eligible to purchase orphan drugs under a federal drug discount program known as 340B.

The 340B program requires drug manufacturers to provide steep discounts to certain hospitals on covered outpatient drugs. While the program originally applied to hospitals that treated a large number of poor, uninsured patients, the Affordable Care Act of 2010 expanded eligibility to other providers, including free-standing cancer hospitals, critical access hospitals, and rural referral and sole community hospitals. At the same time, however, the healthcare law also restricted the ability of those hospitals to purchase orphan drugs at the 340B discount prices.

In its final rule, HRSA said it aimed to balance hospital's ability to access orphan drugs at reduced costs with drugmakers' financial incentives to develop new treatments for rare conditions. It carved out a compromise.

The newly eligible hospitals can purchase the orphan drugs at 340B discount prices, according to the final rule, but only if the drug is used to treat conditions other than the rare disease for which the drug received its "orphan" designation.

PROS AND CONS

While some welcomed the rule, others had reservations.

Safety Net Hospitals for Pharmaceutical Access, an industry group of around 1,000 hospitals, said in a statement that the final rule would help its members serve the nation's most vulnerable patients by improving access to the drugs.

Pharmaceutical industry groups, however, have continued to voice concerns - starting when the HRSA first unveiled its proposed rule in May 2011 - about the risk that such a solution could be seen as off-label promotion.

Even though doctors can prescribe drugs for off-label uses not approved by the FDA, it is illegal for drug companies to market their products for off-label uses. Pfizer Inc's Wyeth unit, for example, on Tuesday agreed to pay over $490 million to settle allegations that it promoted a kidney-transplant drug for unapproved uses in other transplant patients.

If a drug is only approved for an orphan use, the drugmaker would, under the final rule, be required to offer its product to a hospital at a discounted price, knowing full well that the hospital would be using it for a non-orphan, or off-label, use.

"Given the intense scrutiny that FDA, the HHS Office of Inspector General, and the Justice Department give to activity that surrounds off-label uses of drugs, the application of the ceiling price requirement to a manufacturer's knowing sale for off-

label use could cause significant unintended consequences for that manufacturer," said the Biotechnology Industry Organization in a July 2011 comment to HRSA on the proposed rule.

'SIGNIFICANT BLIND SPOT'

The group urged HRSA not to require manufacturers to sell orphan drugs at 340B prices where the drug is only approved for an orphan use.

Pharmaceutical Research and Manufacturers of America, a lobbying organization for drug companies, raised the same concern in its comments to HRSA.

"In the current environment, manufacturers certainly could be concerned about how enforcement authorities could perceive their fulfillment of such orders," said Alice Valder Curran, a lawyer at Hogan Lovells who represents pharmaceutical companies.

She said that although manufacturer groups raised the off-label issue in their comments, the final rule did not address their concerns in any way.

"That is a significant blind spot," she said. "The whole point of the rule-making process is for the agency to respond to stakeholder concerns, and here the agency appears to have ignored this issue completely."

An HRSA spokesman confirmed that those issues were not addressed by the final rule, but stressed that hospitals will have to be able to show that they are using an orphan drug for a non-orphan indication to receive the discount.

Ellyn Sternfield, a healthcare lawyer at Mintz Levin who previously worked as a state prosecutor pursuing healthcare fraud cases, said the final rule could be seen as endorsing off-label use. At the same time, however, hospitals use the discounted drugs to treat not only uninsured patients but also those covered by Medicare and private health insurance policies. Such policies often limit their prescription coverage to FDA-approved uses, which would discourage off-label prescriptions for those patients, Sternfield said.

---- Index References ----

Company: PFIZER INC; WYETH CORP

Industry: (Hospital (1HO39); Drug Discovery & Development Process (1DR41); Pharmaceuticals (1PH33); Healthcare Services (1HE13); Pharmaceuticals Research & Development (1PH57); Hospital Administration (1HO60); Drug Approval Process (1DR91); Healthcare (1HE06); Pharmaceuticals & Biotechnology (1PH13); Healthcare Service Providers (1HE78))

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