



**Teaming with Entities Outside the
Plasma Protein Therapeutics Sector**

COALITION BUILDING

TO MODIFY THE ORPHAN EXCLUSION
FROM THE ANNUAL PHARMACEUTICAL FEE

BY JAY GREISSING

AS PART OF THE AGREEMENT THE PHARMACEUTICAL RESEARCH AND MANUFACTURERS ASSOCIATION

(PhRMA) reached with the Obama Administration during the health care reform negotiations, Senate Committee on Finance Chairman Sen. Max Baucus (D-MT) included an annual pharmaceutical fee in what became the Affordable Care Act. The Internal Revenue Service (IRS) will assess the fee, which will be treated as an excise tax, on the sales of branded prescription drugs into certain government channels. Although the tax is apportioned by the market share of each product, the Finance Committee staff realized it was necessary to protect continued innovation in the rare disease therapy space and so expressly excluded sales of “orphan drugs” from a manufacturer’s fee liability.

“Orphan drugs” are pharmaceuticals or biologicals that have received “orphan designation” from the U.S. Food and Drug Administration (FDA) when their sponsors filed the new drug application or biological license application. A drug that has received “orphan designation” is eligible for a number of incentives, including seven years of marketing exclusivity, grants, and a tax credit for its clinical testing expenses in bringing the product to market for that particular rare disease indication. The exclusion from the annual pharmaceutical fee inexplicably requires the manufacturer to have taken this orphan drug tax credit, rather than to have merely obtained “orphan designation” for its rare disease therapy.

The existing regulatory framework for obtaining “orphan designation” has effectively made it impossible for many plasma protein therapies, despite being exclusively FDA-indicated for the treatment of rare diseases, disorders, and conditions, to have ever taken


the orphan drug tax credit as required for the exclusion from the annual pharmaceutical fee. For a previously unapproved drug or for a new orphan indication for an already-marketed drug, the process of obtaining an orphan designation is relatively straightforward. Generally, the sponsor or manufacturer must only demonstrate that the product will satisfy the rare disease threshold of treating less than 200,000 patients in the U.S. The subsequent brands in the same therapeutic class seeking approval for the same rare disease indication must demonstrate “clinical superiority” to the first-to-market brand in terms of safety or effectiveness, or demonstrate it is making a “major contribution to patient care.” Because of this high threshold, FDA has rarely granted orphan designation to these second-to-market therapies. As a result, plasma protein therapies represent 33 of the 41 drugs identified that are exclusively FDA indicated for the treatment of rare diseases, disorders, or con-

ditions, but lack an "orphan designation" from the Agency.

There is a coalition forming with drug manufacturers outside of the plasma protein therapeutics industry to seek an amendment to the current orphan drug exclusion. Companies like Cephalon, Endo, Shire, Celgene, Millenium, and AstellasPharma U.S. are supportive of PPTA's policy proposal to expand the orphan drug exclusion to also exclude drugs approved or licensed by FDA for marketing solely for one or more rare diseases or conditions. The interest of these companies primarily lies with the decision by the Finance Committee to hinge the exclusion on the orphan drug tax credit and the interpretation of the IRS that the manufacturer had to have actually taken the credit, not merely qualify for it.

Many drug manufacturers were either unable to take the credit (for reasons beyond failing to obtain orphan designation), or made the business decision to take a more attractive credit. For example, the tax credit was not available for an 18 month period in the late 1990s. Prior to June 1, 1997, the Orphan Drug Act tax credit was not permanent. Between January 1, 1995 and June 30, 1996, Congress had failed to reauthorize this provision, making it impossible for manufacturers to claim the credit for clinical testing expenses incurred during that period. Additionally, most drug manufacturers did not claim the tax credit during the 1980s and early 1990s because they were receiving special tax breaks for having established manufacturing operations in Puerto Rico. More than 40 of

the world's largest drug manufacturers created thousands of jobs in Puerto Rico during this period in return for a tax exemption for all income derived from the specified facility. Manufacturers that elected the tax credit for doing business in Puerto Rico in a given year could not also claim the orphan drug tax credit for any qualifying clinical testing expenses incurred during that same taxable year. It is also important to note that during the first 12 years of the Orphan Drug Act, new market entrants were unlikely to claim the orphan drug tax credit because manufacturers could not carry unused credits forward or backward; thus, initially under the law, manufacturers had to have income and high enough tax liability to take the orphan drug tax credit, which was difficult for newer market entrants that lacked revenue. Finally, some manufacturers may choose to claim the research and development tax credit, rather than the orphan drug tax credit for its clinical testing expenses because they are unable to claim both credits for the same qualifying clinical testing expenses.

Regardless of the rationale, a broad coalition supporting the modification of the orphan drug exclusion is vital to its success. PPTA will strongly advocate for this policy that will reward past and encourage future innovation in developing therapeutic interventions for the treatment of rare diseases, disorders, and conditions. 

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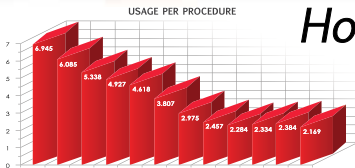


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