Solving the Orphan Drug Problem

"Motherhood, the Flag, and apple pie" need to move over to make room for another revered entity. "Orphan drugs" constitute the newest member of this select group.

Every government official, every drug firm, every consumer representative, and every health care organization seemingly has a policy position or other means of indicating their support for research, development, and marketing of drugs of little commercial value, otherwise popularly known as "orphan drugs."

Moreover, various bills on the subject have been introduced in the U.S. Congress; the Food and Drug Administration has established a separate unit within the Commissioner's Office on Orphan Product Development; the Pharmaceutical Manufacturers Association has set up a blue-ribbon Commission on Drugs for Rare Diseases; and there has been a host of other activities ranging from congressional hearings to lay audience-prime time television shows.

Indeed, things seemed to be moving along in fine fashion insofar as solving the "orphan drugs" problem. The PMA's Commission wasted no time in getting itself off and running. In mid-June it announced that it had made its first selection: *"At its recent meeting on June 10, 1982, the PMA Commission on Drugs for Rare Diseases unanimously recommended further development of L-5-hydroxytryptophan (L-5HTP) in the treatment of post-hypoxic myoclonus."* Accompanying the announcement was an array of impressive information disclosing the scientific and medical considerations that went into the selection process, the expertise of consultants who participated, and the importance of the drug chosen and its value in treatment of the disease involved.

Moreover, the regulatory wheels over at FDA also seemed to be moving with rather unaccustomed speed. A statement from the FDA's office of Orphan Products Development outlining the NDA approval requirements was released simultaneously with the PMA Commission's announcement. Finally, a summary report was included that described the disease, myoclonus, and the reasons why the group felt that this disease and this drug clearly were their first choice in launching what we might refer to as "the war on orphan drugs."

So far, so good. We all had reason to believe that a great start had been made and concrete progress would be right around the corner.

But not so.

An "Orphan Drug Update" newsletter, published by the National Coalition for Rare Disorders, carried in its September 1982 issue an article entitled **"L-5HTP NOT ADOPTED!!"** The article described the situation as follows:

"However, despite the (PMA) Commission's massive effort to publicize the need for a sponsor of L-5HTP (more than 2,000 announcements were distributed by PMA), not one PMA member has stepped forward *to give hope to people with Myoclonus . . . A small generic manufacturer is interested (not a PMA member), but a final commitment has not been made by the manufacturer.*

"Consumers must question why none of the multi-million dollar pharmaceutical corporations stepped forward to adopt L-5HTP. Why would a small manufacturer, who has much more to lose, offer to adopt this drug? At press time, we are still in doubt as to the future of L-5HTP, and people with Myoclonus are suffering needlessly. Since the government and industry claim they will both solve the orphan drug dilemma 'voluntarily,' why are people with Myoclonus without their therapy? In this instance, HHS, FDA, NIH and the PMA have all failed to live up to their promises! In the absence of a legislative mandate, the Coalition feels that the saga of L-5HTP will be repeated again and again by other drugs for a great variety of orphan diseases."

More recent information appears to confirm that Bolar Laboratories—which has been described in the pharmaceutical press as a small, generic, non-PMA member drug company—is *"assuming the responsibility for financing clinical trials on the product (L-5HTP) and the administrative role of shepherding an NDA through FDA."*

Hence, it appears that a fairy godmother, or godfather, has been found for this particular agent. But although this immediate crisis has passed, what about the second orphan drug selected? and the third? and the next after those? What firm, if any, will step forward to undertake the financially unrewarding, but humanely necessary, task of sponsoring those agents through the drug approval process?

When the cry went up for patient information on prescribed drugs, FDA responded by proposing mandatory Patient Package Inserts (PPIs). But that proposed regulation was withdrawn because the health professions—and pharmacy and medicine particularly—moved decisively to embrace voluntary systems of patient education and information regarding prescribed and dispensed drugs.

Similarly, it appears to us that the drug industry now is faced with comparable options. Everyone else, including the industry's own trade association (PMA), has done all they can do to solve the orphan drugs problem. Whether the voluntary approach will work depends on individual drug companies making the necessary financial commitment.

If individual companies fail to respond in a positive fashion, legislation—and with it regulations, government intrusion, red tape, and all the other things that run contrary to the free enterprise system—is bound to ensue. But is the industry, on a company-by-company basis, willing to pay the relatively small price of voluntary service in order to retain its freedom of operation in this area?

—EDWARD G. FELDMANN
American Pharmaceutical Association
Washington, DC 20037

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