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POOR LITTLE RICH DRUGS?

by Howard M. Metzenbaum

The sole purpose of the Orphan Drug Act is to encourage a company to develop a drug of little commercial value for the victims of a rare disease. Primarily through the government guarantee of a seven-year monopoly, the act has spurred the development of 60 orphan drugs for 74 different rare diseases. These include breakthrough drugs that prevent and treat respiratory distress syndrome in infants and a rare disease that causes blindness.

Tragically, the act has also allowed a handful of profiteers to use their seven-year monopoly as a shield to block competition and charge absurdly high prices for blockbuster orphan drugs. Genzyme charges up to $350,000 for Ceredase, an orphan drug for Gaucher's disease. Likewise, Genentech and Eli Lilly charge between $10,000 and $30,000 for human growth hormone, an orphan drug for severe growth hormone deficiency. At these prices, ask yourself how many American families can afford these miracle drugs?

The Kassebaum/Metzenbaum bill will encourage price competition for blockbuster orphan drugs by allowing competitors on the market when sales of an orphan drug reach $200 million. It will not halt the sale of the first drug or tell a company what it can charge. This effective approach to bringing down the price of blockbuster orphan drugs is supported by, among others, the National Association for Rare Disorders, which represents 127 rare disease groups, the National AIDS Commission, the Association of Biotechnology Companies (ABC) and small biotechnology firms like Immunogen.

As you might expect, a number of large and powerful drug companies oppose any changes to the Orphan Drug Act. They claim that our bill will be the death knell for the program. But just nine years ago when the original act was being debated, Pharmaceutical Manufacturer's Association (PMA), the industry's trade association, opposed the act, telling us that it was totally unnecessary for the development of orphan drugs. However, now that some of their members are making a windfall under the act, the PMA makes the unbelievable claim that changes to this once "unnecessary" program would chill the development of orphan drugs.

Incredibly, the PMA even claims that legislation to change the act has caused a "disturbing" decline in orphan drug applications in 1991. That is nonsense and the PMA knows it.

The Food and Drug Administration has documented that in 1990 there was a flood of orphan drug applications from companies trying to take advantage of the prospective application of pending legislation. That unanticipated deluge artificially inflated the total number of applications for 1990. If you eliminate the record number of hastily slapped-together 1990 applications that had to be withdrawn from consideration, the number of orphan drug applications filed in 1991 has not appreciably changed.

Opponents also claim that they won't be able to recoup their research and development costs under our $200 million competition trigger. That is nonsense and also untruthful. Research and development costs for high-priced blockbuster drugs ran between $10 and $45 million for all but one company. And that company came in at $150 million, well below the $200 million competition trigger.

An equally ingenuous claim by opponents is that a competition trigger will dry up investment in biotechnology. That claim is refuted by the fact that the ABC strongly supports the competition trigger. The ABC represents innovative small- and mid-sized biotechnology companies that are heavily involved in orphan drug development.

One of the nation's leading investment banking firms has publicly assured ABC that our bill would not affect capital investment in biotechnology. That advice makes perfect sense when you consider that our bill allows the industry to guarantee investors a $200 million market totally free of competitors.

Our bill will not affect, much less diminish, orphan drug development. Only 3 percent of the drugs designated as orphans are now or are expected to become blockbusters. So, the amazing commercial success of such orphan drugs as Humatrope, Protropin, Epogen, NebuPent, and Eldepryl simply cannot be what motivated the development of the other 97 percent.

Moreover, it is clear that companies like Genentech did not rely on the Orphan Drug Act to develop their blockbusters. Documents obtained by my Antitrust subcommittee show that Genentech began work on human growth hormone in 1979 and received approval in October, 1985. But it wasn't until the Act was amended in August, 1985, to include patentable drugs, that human growth hormone even became eligible for the orphan drug program. Clearly, Genentech never relied on the Orphan Drug Act's protection to bring its blockbuster to market.

Moreover, what right thinking company would walk away from a drug with a $200 million sales potential even if it had to face competition down the road? The biggest opponent of our bill, Genentech, has made it clear that it will go ahead with the development of DNase, its potential blockbuster for cystic fibrosis. The reason is clear—the drug promises annual sales of $270 million or more in five years.

We cannot ignore the fact that high prices are making life-saving and hope-giving orphan drugs practically unaffordable to an increasing number of American families. I believe that Senator Kassebaum and I have the answer to the problem in our bill. Terminating a company's seven-year market monopoly when its sales reach $200 million will encourage price competition and make orphan drugs much more affordable.

The opinions expressed are the author's own and not necessarily those of **Bio/Technology**.

Howard M. Metzenbaum is U.S. Senator (D.) from Ohio. Along with U.S. Senator Nancy Kassebaum (R.) of Kansas, he is the sponsor of a bill amending the Orphan Drug Act of 1983. This column is based on his opening remarks at hearings on these amendments in March.