

## **RARE DISEASE DAY FACT SHEET: THE *ORPHAN DRUG ACT***

The *Orphan Drug Act* was approved by the two houses of Congress in 1982 and signed into law by President Ronald Reagan in 1983. Although few people realized its significance at the time, it has become one of the most important and successful pieces of healthcare legislation today.

### **What is an Orphan Drug?**

This term refers to a drug or biologic (such as a vaccine or blood product) that treats a rare disease or condition. A disease is considered rare if fewer than 200,000 people in the United States have it.

Orphan drugs may be new products or they may be drugs already approved for other diseases that are later found to treat a rare disease. A drug or biologic becomes an orphan drug when the sponsor applies for an orphan drug designation, and the drug is then designated by the Food and Drug Administration (FDA).

Like every other drug, orphan drugs must go through the normal FDA approval process and be evaluated for safety and effectiveness for the intended use. To date, more than 1,700 drugs and biologics have been designated as orphan drugs since 1983. Nearly 330 have been approved for the U.S. market by FDA.

### **How the Orphan Drug Act Has Helped**

Discovering and developing a new drug can be very expensive. Since rare diseases affect relatively small numbers of people, companies would not be able to recover this expense without the incentives provided by the *Orphan Drug Act*. Those incentives are:

- A seven-year period of marketing exclusivity after an orphan drug reaches the American market
- Tax credits for the costs of clinical research
- Opportunities to apply for grant funding for certain clinical testing expenses
- Assistance in clinical research study design
- Waiver of Prescription Drug User Fee Act (PDUFA) filing fees (fees that companies normally pay to FDA)

### **The *Orphan Drug Act* was a Bipartisan Effort**

The *Orphan Drug Act* was introduced originally by Senator Nancy Kassebaum (R-KS) and Representative Henry Waxman (D-CA). It was cosponsored by several current members of Congress, among them Senators Orrin Hatch (R-UT) and Edward Kennedy (D-MA).