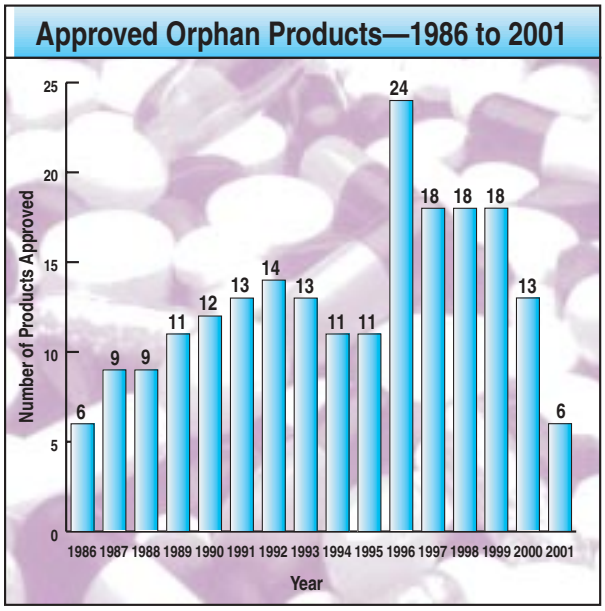
FDA Fights Rare Diseases:
New Help for Patients Without Treatments

Years ago, people diagnosed with a rare disease might have found themselves with little hope for a medical treatment. This changed in 1983 when Congress passed the **Orphan Drug Act,** a law that offered drug companies special incentives to develop products for treating diseases with fewer than 200,000 patients a year. The inducements include seven-year marketing exclusivity, tax credit for the product-associated clinical research, research design assistance by the FDA, and grants of up to $300,000 per year.



Thanks to the drug sponsors' gratifying response to this program, which can be applied to up to 6,000 rare diseases, the FDA has so far approved more than 200 so-called "orphan" drugs.

Here are some of the rare diseases that now can be treated with "orphan" medications the FDA approved in recent years:

- **Sickle cell anemia**, an inherited blood disorder that causes chronic anemia and pain.
- **Cystic fibrosis,** an inherited disorder affecting children and young adults.
- ***Pneumocystis carinii* pneumonia**, an infection that strikes high-risk, HIV-infected patients.
- **Hansen's disease (leprosy),** a disease that attacks the skin and nervous system.
- **T-cell lymphoma**, a type of blood cancer.

Other rare diseases that now have an orphan treatment include ovarian cancer, Lou Gehrig's disease, neonatal respiratory distress, hemophilia, organ transplant rejection, Huntington's disease, and juvenile rheumatoid arthritis.

Humanitarian Use Devices

The success of the orphan drug program encouraged congressional authorization in 1997 for the FDA's humanitarian use device program, which offers incentives for the development of medical devices for patient populations too small to justify full-scale product evaluation. Devices approved under this mandate require only evidence that their probable health benefit is greater than the risk of use, a standard which is less costly to achieve than the level of safety and effectiveness required for regular devices. The FDA approvals under this program include a stent to treat urinary tract obstruction in unborn babies and a cardiac patch for repair of holes in the heart—products that would not be available without this program.

For more information, please contact the FDA's Office of Orphan Product Development at 301-827-3666 or 1-800-300-7469.

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