

A DECADE OF Innovation

Advances in the Treatment of Rare Diseases



Over the past decade (1995–2005), biopharmaceutical companies have developed over 160 drugs to treat rare or “orphan” diseases that affect 200,000 or fewer Americans. This number is up from 108 the decade before and just 10 in the 1970s. These advances bring hope to many; there are 6,000–7,000 rare diseases affecting a total of 25 million Americans.

Among the new treatments developed in the last decade are:

- Three new medicines approved to treat respiratory distress syndrome, which affects 50,000 premature babies every year in the United States.
- The first drug to treat the cause of Fabry disease rather than just its symptoms. Fabry disease is a potentially fatal disorder in which fats are not stored properly in the body causing burning sensations and other symptoms.
- A trailblazing targeted therapy that dramatically improves survival for many patients with chronic myeloid leukemia.



Biopharmaceutical companies continue the quest to develop treatments for rare diseases. A record number of new orphan drugs are in development according to the latest data from the FDA. Scientists are working everyday to get these new treatments to patients with rare diseases.



Find out more and read the full publication *A Decade of Innovation: Advances in the Treatment of Rare Diseases* at www.phrma.org and www.innovation.org.