



spotlight on health

New Medicines Available To Treat Rare Diseases

(NAPSA)—Since 1995, America's pharmaceutical research companies have made great strides in fighting rare diseases. More than 160 new medicines to treat rare or "orphan" diseases have been approved by the Food and Drug Administration (FDA) in the last decade alone.

According to the National Institutes of Health, more than 6,000 rare diseases afflict a total of 25 million Americans. And the FDA says 85 to 90 percent of rare diseases are serious or life-threatening, making the search for new treatments and cures all the more important.

"America's pharmaceutical research companies have made incredible progress in finding new medicines that offer hope to patients with rare diseases," says Billy Tauzin, president and CEO of the Pharmaceutical Research and Manufacturers of America (PhRMA). "Continuing innovation by these companies is the key to developing new medicines and offering patients a chance for a brighter, healthier future."

"A Decade of Innovation: Advances in the Treatment of Rare Diseases"—a new report issued by the National Organization for Rare Disorders, the Genetic Alliance and PhRMA—highlights some of the many important drugs for rare diseases that the FDA has approved in the past decade. These medicines have helped patients lead very different lives than they would have just a few years ago. For instance:

- Rilutek® (riluzole) is the first drug to show any increase in survival among patients with amy-



Great strides have been made in developing treatments for rare diseases.

trophic lateral sclerosis, or ALS.

Also known as Lou Gehrig's disease, ALS is a progressive disease that affects motor nerve cells in the brain and spinal cord, eventually causing the brain to lose its ability to control muscle movement.

- Fabrazyme® (agalsidase beta) is the first drug to attack Fabry disease at its root. Patients with the condition—mostly men—are deficient in an enzyme involved in the breakdown of fats, causing a buildup of fats in their blood vessels and organs. These patients often survive to adulthood, but are at increased risk for stroke, heart attack and heart disease.

While researchers have made significant progress in the fight against rare diseases, they continue working to find more new treatments for people with these rare conditions. Medicines for patients with Crohn's disease, chronic myeloid leukemia, pulmonary arterial hypertension and many more rare diseases are currently in development.

To view the "Decade of Innovation" report, visit www.phrma.org and www.innovation.org.