spotlight on health

New Hope For Sufferers Of Rare Diseases

(NAPSA)—A number of new medicines have been approved in recent years to help patients suffering from common conditions such as arthritis, diabetes or heart disease. But for millions of Americans diagnosed with a rare disease—defined as a condition affecting fewer than 200,000 patients in the United States—treatment options can be limited.

Fortunately, real progress in creating new treatments for rare diseases has been made in the last few years. Several new medicines (known as orphan drugs) have now been approved by the U.S. Food and Drug Administration (FDA) to treat rare diseases such as Pompe disease, myelodysplastic syndromes, enzyme deficiencies and rare cancers. In fact, a new report by the Pharmaceutical Research and Manufacturers of America (PhRMA) finds that there are 303 medicines currently in human clinical trials or awaiting approval by the FDA for more than 600 rare diseases. This compares to 133 medicines in development in 1989 and 189 in 1992.

Since 1995, more than 160 medicines have been approved to treat rare diseases, compared to 108 in the 1980s and fewer than 10 in the 1970s. Advances in science—such as achieving a better understanding of molecular and genetic causes of disease—have helped increase treatments. The Orphan Drug Act of 1983, which provided tax relief and some marketing exclusivity for companies that developed orphan drugs, helped as well. Under the act, 1,679 medicines have been designated as orphan drugs.

A Closer Look At Rare Disease

The National Institutes of Health estimates that there are 6,000 rare diseases affecting 25 million Americans. A major area of research in rare diseases is cancer. Rare cancers, such as liver, pancreatic and thyroid, account for more than one-third of all rare disease research, with 133 medicines in development to treat these conditions.

Hundreds of drugs are being developed to treat rare diseases.

Other important areas of research include neurologic disorders (such as multiple sclerosis and muscular dystrophy), with 35 medicines in development; infectious diseases (such as anthrax and West Nile virus), with 28 medicines in development; respiratory diseases (such as cystic fibrosis), with 22 medicines in development; and genetic disorders, (such as sickle cell disease), with 16 medicines in development.

"Biopharmaceutical research is entering an exciting new era with our growing understanding of the genome and powerful scientific research tools," explains Billy Tauzin, president and CEO of PhRMA. "Based on the number of orphan drugs in the pipeline, the number of treatments available to patients with rare diseases is expected to rise in the coming years."

For more information, visit www.PhRMA.org.