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More than 160 Medicines to Treat Rare Diseases Approved in Last Decade

Washington, D.C. (April 20, 2006) — Over the last decade, America's pharmaceutical research companies have made great strides in fighting rare diseases, with more than 160 new medicines to treat rare or "orphan" diseases having been approved by the FDA, according to a new report issued today by the Pharmaceutical Research and Manufacturers of America (PhRMA), National Organization for Rare Disorders (NORD) and the Genetic Alliance (GA).

"America's pharmaceutical research companies have made incredible progress over the past decade in finding new medicines that offer hope to patients with rare diseases," said Billy Tauzin, President and CEO of PhRMA. "Continuing innovation by these companies is the key to developing new medicines and offering patients a chance for a brighter, healthier future."

The new PhRMA report, "A Decade of Innovation: Advances in the Treatment of Rare Diseases," was released at a press conference in Washington, DC this afternoon. The report highlights some of the many important drugs for rare diseases that have been approved in the last decade, many of which have allowed patients to lead very different lives than they would have just a few years ago.

For instance, after 126 years, a breakthrough treatment—Rilutek® (riluzole)—was approved for treatment of amyotrophic lateral sclerosis, or ALS, in 1995. ALS, also known as Lou Gehrig's Disease, 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. It eventually leads to death because it often affects the muscles needed for breathing. Riluzole is the first drug to show any increase in survival for ALS patients, according to the report.

The report also describes a significant advance in the treatment of Fabry disease. Fabry disease patients—mostly men—are deficient in an enzyme involved in the breakdown of fats, causing a buildup of fat in their blood vessels and organs. These patients often survive to adulthood, but are at increased risk of strokes, heart attack and heart disease, digestive problems, and kidney failure. Fabrazyme® (agalsidase beta), approved in 2003, is the first drug to attack Fabry disease at its root rather than just ease its symptoms.

According to the National Institutes of Health, more than 6,000 rare diseases affect a total of 25 million Americans. One in every 10 Americans receives a diagnosis of rare disease. And, according to the FDA, 85-90% of rare diseases are serious or life-threatening, making the search for new treatments and cures all the more important.

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“The National Organization for Rare Disorders is grateful to PhRMA for focusing this spotlight on rare diseases,” said Diane Dorman, vice president, public policy of NORD. “*Decade of Innovation* doesn’t just describe a period of stunning medical advances, it documents a brief window in time during which people without hope learned to believe again that they might have a future.”

“We applaud the many talented individuals—particularly those who are part of PhRMA’s member companies—who devote their time, energy and resources to search for treatments and cures for rare diseases,” added Sharon Terry, president and CEO of Genetic Alliance.

Although researchers have made significant progress over the past decade, they continue to work to find more new treatments for patients with rare diseases, according to the report. Medicines for patients with Crohn’s disease, chronic myeloid leukemia, pulmonary arterial hypertension, and many more rare diseases are currently in development.

“Anyone who suffers from one of these diseases or who has an afflicted loved one knows the importance of these medicines and this research,” Tauzin added. “And each advancement in the treatment of a rare disease gives patients a precious gift—hope for the future.”

To view the report, please go to www.phrma.org.

The Pharmaceutical Research and Manufacturers of America (PhRMA) represents the country’s leading pharmaceutical research and biotechnology companies, which are devoted to inventing medicines that allow patients to live longer, healthier, and more productive lives. PhRMA companies are leading the way in the search for new cures. PhRMA members alone invested an estimated \$39.4 billion in 2005 in discovering and developing new medicines. Industry wide research and investment reached a record \$51.3 billion in 2005.

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