



spotlight on health

Tackling Rare Diseases That Affect Millions

(NAPSA)—The estimated 25 million Americans who suffer from one of the 7,000 identified rare diseases may be relieved to know biopharmaceutical researchers are working on over 400 new medicines to help fight those conditions.

The Challenge

Roughly 80 percent of rare diseases affect fewer than 6,000 patients in the U.S. There is a great need for new medicines to treat these diseases, most of which afflict children. However, the challenges of finding new treatments can be equally great. Many of these diseases are little understood and it can take years just to get an accurate diagnosis, which also limits the pool of potential clinical trial participants. That can make it difficult to enroll enough clinical trial participants to meet the Food and Drug Administration's requirements. Because of this and other hurdles, rare disease research can be significantly more challenging and time consuming than other biopharmaceutical research, which can generally take 10 to 15 years and cost, on average, \$1.3 billion to bring a new medicine to patients.

Some Solutions

Meanwhile, positive policies such as the Orphan Drug Act help spur innovation by providing fair incentives to companies that accept the burden, cost and risk of entering this field of research. Says Chris Silber, M.D., vice president of U.S. Clinical Research and Medical Affairs for Lundbeck, Inc., "Finding



Orphan drug research can bring a first-of-its-kind medicine to patients and give them hope for the future.

a therapy that really works in an underserved population that has been through tremendous adversity is one of the most gratifying things I can imagine."

"Once you've talked to a desperate parent whose child is suffering or dying, you will be forever convinced of the need for medical innovation, including for terrible diseases that afflict even just a few," adds PhRMA President and CEO John J. Castellani. "Researchers at biopharmaceutical companies are working every day to bring hope to patients with limited options." PhRMA represents the country's leading pharmaceutical research and biotechnology companies, which are devoted to inventing medicines that allow patients to live longer, healthier lives.