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APS FOUNDATION OF AMERICA, INC. JOINS WITH NORD TO CELEBRATE THE 25th ANNIVERSARY OF THE ORPHAN DRUG ACT

La Crosse, WI, January 3, 2008----On January 4, 1983, President Ronald Reagan signed groundbreaking legislation that brought real hope for more than 25 million Americans living with one of the 7,000 rare disorders recognized today. The *Orphan Drug Act of 1983* (ODA) spurred breakthrough drug research and development for little-known diseases, while providing a potent catalyst to the growth of the pharmaceutical and biotechnology industries in the U.S.

An "orphan" disease is defined by the U.S. Food and Drug Administration (FDA) as a disease or condition that affects fewer than 200,000 Americans. In the past, because of very low prevalence, orphan diseases were overlooked by drug and medical device developers. In the 10 years prior to passage of the ODA, only 10 new drugs for rare diseases were developed by the pharmaceutical industry. In the 25 years since the approval of the ODA, more than 300 new orphan drugs have been approved in the U.S.—an average of about 11 new drugs every year.

The APS Foundation of America, Inc. is just one organization that acts on behalf of patients and their healthcare professionals to promote improvements in diagnosis and treatment for a rare disease—Antiphospholipid Antibody Syndrome (APS). APS affects new patients every year. Women are more likely than men to be affected by APS. Some estimates say that 75% to 90% of those affected are women. For example, it has been estimated by some doctors that one third of all of young strokes (defined as under the age of 50) are due to APS.

In obstetrics it is estimated by some doctors that up to 25% of all women with 2 or more spontaneous miscarriages have APS. Some doctors believe that 1 in 5 of all Deep Vein Thrombosis (DVTs), Pulmonary Embolisms (PEs), and even worse, amputations are due to APS. And it is believed that 40-50% of patients

with Lupus also have APS. Still, with these statistics, APS rarely is discussed as a women's health issue and is misdiagnosed often. Therefore the total number of people affected and true statistics are unknown really.

APS is an autoimmune disorder in which the body recognizes certain normal components of blood and/or cell membranes as foreign substances and produces antibodies against them. There are two known forms of APS. APS may occur in people with systemic lupus erythematosus, other autoimmune disease, or in otherwise healthy individuals. Sadly, when most people hear about APS and it being referred to as autoimmune disease, they incorrectly confuse the terms autoimmune with acquired immune deficiency syndrome (AIDS); or they think this is a form of cancer.

This lack of knowledge and awareness results in needless suffering for persons with APS. Misdiagnosis and / or delayed diagnosis usually result in damage to vital organs. The need to bring a national attention to APS as a common factor in multiple miscarriages, thrombosis, young strokes and heart attacks is vital in order to bring a joint effort to research, funding, early detection, and eventually, prevention and cure for APS.

There is no cure for APS, but there is treatment. The treatment of choice for patients with APS who have had a blood clot is anticoagulant therapy. Please note there is no FDA approved treatment to control these antibodies that we for that cause these problems. This is usually successful in preventing further clots. For women with APS and recurrent miscarriages who have not had a prior blood clot, the use of anticoagulant therapy during the pregnancy significantly increases the likelihood of a successful outcome. Some individuals may have elevated antiphospholipid antibodies but have no clinical manifestations of the syndrome. These individuals are usually treated with aspirin. Aspirin reduces the risk of blood clots by making the platelets less sticky. Studies are ongoing to determine how helpful aspirin is and whether low doses of anticoagulants might be more effective.

In general patients who have had a blood clot (i.e., stroke, heart attack, DVT) and have persistently positive tests for antiphospholipid antibodies should be treated with anticoagulants indefinitely. Discontinuing treatment after a fixed period of time, such as six months, may be quite dangerous in such patients. In some patients with a history of blood clots, antiphospholipid antibodies may disappear after a certain period of time. It is not known whether it is safe to stop anticoagulation in this situation. Consultation with a doctor experienced in treating APS is recommended for such patients.

On the 25th anniversary of the signing of the *Orphan Drug Act,* we join with the National Organization for Rare Disorders (NORD) and its members in continuing our commitment to, and support for, patients around the world with rare diseases. We also celebrate the success of this legislation in bringing improvements in healthcare to millions of Americans. Through drugs as diverse as Thalomid® (thalidomide), Myozyme® (alglucosidase alfa), Nutropin® (somatropin {rDNA origin} for injection), and Gleevec® (imatinib), the ODA has offered new opportunities for patients that might never otherwise have existed.

NORD was founded in 1983 to help people with rare "orphan" diseases and to assist the organizations that serve them. The founders of NORD were a driving force behind the passage of the ODA. This is an appropriate time to congratulate them on their vision and recognize them for their persistence and their success.

We also recognize that the achievements of companies like Genentech, Amgen, Genzyme, Allergan, Cephalon, Celgene and others have been profoundly influenced by the existence of the ODA, which has brought jobs for thousands in the biotech and pharmaceutical industries, financial rewards for many of the early investors in such companies, and diverted the focus of scientific inquiry to chronic and life-threatening diseases that were previously untreated, leaving patients hopeless, until the ODA was enacted.

With the current advances in such areas as genomics, proteomics, targeted therapeutics, personalized medicine, and stem cell biology, we can expect to see a vast array of new breakthrough products developed for rare disorders in the next 25 years—and we need to make sure that such products will be available and accessible for the patients with these disorders who so badly need them.

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